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DHEW's RESEARCH PLANNING PRINCIPLES: A REVIEW

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NOTICE

The project that is the subject of this report was approved by the Governing Board of the National Research Council, whose members are drawn from the Councils of the National Academy of Sciences, the National Academy of Engineering, and the Institute of Medicine. The members of the committee responsible for the report were chosen for their special competencies and with regard for appropriate balance.

This report has been reviewed by a group other than the authors according to procedures approved by a Report Review Committee consisting of members of the National Academy of Sciences, the National Academy of Engineering, and the Institute of Medicine.

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Dr. Donald Nutter and Gloria Ruby contributed to the drafting of the Report and to assuring that it include the perspectives of all Committee members.

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To Pat Cornwell, I would like to express deep appreciation for her patience and hard work in the preparation and typing of the Report.

Sarah Spaght Brown
Division Director and
Study Director

PREFACE

Background

In late April of 1978, Secretary Califano delivered a major address before the Annual Meeting of the American Federation for Clinical Research, in which he announced his intent to have the Department of Health, Education and Welfare (DHEW) develop by the Fall of 1979 a "comprehensive five-year research plan" for the health-related agencies in the Department.

The plan is to cover the budget period of FY 1982-87, and is to:

- define the health research needs in each of DHEW's health-related agencies, and set overall priorities among these needs;
- define the responsibilities of these agencies for sponsoring or conducting the needed research; and
- provide budget estimates for DHEW activities in health research for the five-year period encompassed by the plan.

In the early summer of 1978, the National Institutes of Health (NIH) was charged with the responsibility for organizing the development of the plan. Other agencies are involved in the planning activity because they participate in supporting, planning, or performing some aspects of health research.

These agencies are the:

Alcohol, Drug Abuse, and Mental Health Administration (ADAMHA)

Center for Disease Control (CDC)

Food and Drug Administration (FDA)

Health Care Financing Administration (HCFA)

Health Resources Administration (HRA)

Health Services Administration (HSA)

Office of the Assistant Secretary for Planning and Evaluation, Office of the Secretary (OASPE)

Office of Health Research, Statistics, and Technology, which includes the National Center for Health Services Research (NCHSR), the National Center for Health Statistics (NCHS) and the National Center for Health Care Technology (NCHCT)

The plan is being developed through a two-step process. The first activity is the formulating of broad principles that will underlie the research plan; the second is the drafting of the plan itself. The goal is to issue a plan in the Fall of 1979 to guide the health research activities of DHEW over a five-year period, beginning with the 1982 budget cycle.

The first step of developing "research planning principles" itself has had several stages. Initially, each of the DHEW agencies and offices engaged in health research developed its own list of proposed principles in consultation with its respective committees and constituents. These principles were compiled and integrated by NIH, and then reviewed by the submitting agencies and offices. Next, comments on the principles were invited from approximately 1,000 professional societies and health organizations and from other federal departments and agencies. The principles were then revised in light of this external review.

The next stage was to present the suggested principles to a national conference held on October 3-4, 1978, in Bethesda, Maryland. This conference was convened by DHEW, through NIH, to obtain additional consideration of the draft principles. The conference was attended by representatives of the scientific community, public organizations, government leaders, and other interested individuals. At the opening session of the conference, Secretary Califano emphasized the need for research priorities and strategies at a time of limited resources for health research.

The rest of the two-day conference was devoted to panel workshops in five areas: fundamental research, clinical applications and health services research, health promotion and regulation, research capability, and unifying concepts. For each of these areas, a panel of about 20 experts heard statements on the principles from individuals and groups that had requested an opportunity to testify. Each panel discussed and amended the DHEW draft and then developed a report commenting on both the conference and the principles. These reports were presented orally at the closing session of the conference, and then submitted in writing to NIH in November.

During the first week of December 1978, NIH released for comment a revised draft of the DHEW health research planning principles. This most recent version is in Part III of this Report. These revised principles represent the combined suggestions of non-government health organizations and professional societies, other federal departments and agencies, participants who provided testimony at the national conference in October, the panel reports of the October conference, and the suggestions of the DHEW agencies and staff offices supporting or performing health research.

The Institute of Medicine Report

This Report is an independent review and analysis of the December 1978 version of principles, and was developed by a Committee of the Institute of Medicine at the request of DHEW. The Committee assembled for this study attended the October conference described above, and reviewed successive drafts of the principles issued over the last several months. After DHEW receives and reviews this Report and other public commentary, a final version of the principles will be released in the Spring of 1979.

This Report not only discusses the individual principles, but also addresses itself to some broader issues in health science policy, and to the nature and process of this current planning activity.

The Report is in four sections following the Summary:

- Part I discusses the contributions of health science research, and the need for an increased commitment to such research, particularly in light of the current burden of illness and the growth in understanding of the factors that contribute to disease.

- Part II discusses the current planning activity, and some of its potential benefits and liabilities, and also comments on the process being used to develop the plan.

- Part III contains the DHEW draft principles and the Committee's commentary on their themes, strengths, and limitations.

- Part IV includes a list of some major issues in health science policy that the Institute of Medicine Committee finds to be inadequately addressed or absent altogether in the principles, and also notes some research requirements for productive health science planning.

SUMMARY

This Committee strongly endorses the introduction of long-range planning for health research as initiated by the Secretary of DHEW. We believe that health research planning, when well performed, will prove to be a valuable mechanism for developing sound and productive national health research policy. Well conceived and executed planning is the nation's best assurance that there will be optimal allocation of resources for health research, and that precious resources will be utilized efficiently.

The plan and the policies growing from it should take into account the past productivity and future promise of the health sciences; our growing understanding of the many factors that affect health; the essential need for research across the full range of the health sciences to permit further advances in the diagnosis, treatment, and prevention of disease; the heavy burdens imposed on society by illness; and the strong support of the public for health sciences research. The plan should also reflect the challenge and opportunity afforded the U.S. health sciences community to continue providing leadership in the development of knowledge needed to prevent disease and promote health not only in this nation, but in many other nations as well.

We support fully the major message of both the research principles presented to this Committee for review and the October 1978 Conference at NIH during which the principles were discussed: namely, a vigorous reaffirmation of federal support for the health sciences, and a clear call not only for an increased commitment to existing programs of high quality, but also for expansion to important new and neglected areas. In neither the principles nor the conference was the notion advocated of limiting or reducing federal support of health research. Further, we support without reservation another message of the principles and of the conference: that fundamental science is the cornerstone of health research, and that support for new and neglected areas must not erode existing support for fundamental research.

An additional perspective which we endorse is a broadened concept of the health sciences. Rather than equating health research with biomedical research only, the principles recognize the importance of such areas a epidemiology and biostatistics, environmental sciences, health services research, and the behavioral and social sciences as they relate to health and prevention of disease. In addition, we wish to emphasize the importance and promise of the physical and chemical sciences and engineering for advancing our understanding of biological and medical processes and for achieving successful and practical solutions to difficult problems in fundamental biology and in clinical medicine.

We believe that current resources devoted to health research are seriously inadequate in relation both to our society's need for the results of health research and to the highly promising opportunities emerging in the health sciences. In 1977, for example, only about 3.4 percent of total national health expenditures was devoted to health research--a figure which, we are convinced, is insufficient and should be increased, particularly since health care should be soundly based in science. An effective plan for DHEW's support of the health sciences requires that health research be adequately appreciated and valued in the competition for resources, and that the need to build as well as to preserve the capability for this vital work be recognized. We are deeply concerned that excessive emphasis on the limitation of resources for health research may lead to a plan of inferior quality and limited utility.

In taking this view, the Committee recognizes that its position may be dismissed as just another example of special interest pleading. We believe nevertheless that health research merits increased support because of its demonstrated contributions to improving health care, its long-term promise for diminishing the crushing personal and economic burdens of illness, and its enhancement of the quality and duration of life—all of which are of high social priority. We also endorse fully the responsibility of the health sciences community to assure that funds are spent efficiently and prudently, through careful research administration and mechanisms that operate to insure that only scientific projects and investigators which meet very high standards are supported.

We are in substantial agreement with the main thrust of the principles, which are presented and discussed in detail in Part III of this Report. Major themes in the principles which we found particularly significant include:

- the essential contribution of fundamental research to the prevention, diagnosis, and treatment of disease;
- the need for long-term, predictable support for the health sciences;
- the vital role of clinical investigation as a bridge between fundamental research and medical practice, and the need to strengthen this field;
- the need for research on personal and group behavior as significant factors determining the success or failure of programs directed toward health promotion and disease prevention;

- the important role of health services research in studying the structure, processes and effects of the health care system;

- the importance of developing improved, efficient mechanisms for technology transfer and information dissemination to facilitate the applications of research;

- the need for analysis and evaluation of the costs, risks, and benefits of new and existing health care interventions, without impeding desirable clinical innovations;

- the need for interdisciplinary and multidisciplinary programs in the health sciences to address the nation's health problems, many of which are highly complex and require the interaction of many scientific disciplines for their resolution;

- the importance of investigator-initiated proposals as a principal mechanism for research grant funding;

- the critical role of excellence as the ultimate criterion by which research should be supported, and the high value of the peer review system in helping to assure such excellence;

- the essential role of training programs to supply qualified research scientists on a continuing basis to all the health sciences;

- the pressing need to attend to the stability, maintenance, and essential physical renewal of the institutions within which much of health research occurs;

- the need to exercise care when changing research priorities in order to avoid wasteful disruptions and dislocations in scientific programs and institutions;

- the recognition that the policy and health care agencies, and especially the regulatory agencies within DHEW have research needs that are not adequately appreciated and must be attended;

- the recognition that the public needs to be better informed about the concepts and practices of regulation in the health area (such as the regulation of foods, drugs and devices), and the data on which regulatory decisions are based, so that public debate on the relevant issues will be conducted with more knowledge and wisdom;

- the importance of having the scientific community inform the public about the accomplishments derived from public support of the health sciences.

Several important issues are inadequately resolved or are not considered in the principles at all. Some of these include:

- the development of an appropriate balance between the requirements of predictability for research support of well established productive investigators, and the need to provide opportunities for promising but untested young investigators;

- the promotion of productive interaction between public and private health research activities;

- the relationship of health research programs and needs in agencies such as the Veterans Administration, the National Science Foundation, and the Departments of Defense and Energy to health research programs and priorities in DHEW;

- organizational aspects of NIH, including both its intramural and extramural programs, and the strengths and limitations of the categorical institute structure;

- the locus of responsibility within DHEW for fundamental research in areas other than the biomedical sciences; and

- mechanisms by which the research needs of the regulatory agencies in particular are to be met.

Since the principles are very general and occasionally ambiguous, and are not assigned any relative value or priority, they appear to offer little guidance for detailed budgeting, setting priorities, or sorting out the health research missions of DHEW agencies. The value and utility of this planning activity will become clearer when the actual plan, drafted on these broad principles, is eventually developed and available for review.

As regards the process being used to develop the plan, we are impressed by the desire of DHEW, and NIH in particular, to incorporate public review and discussion as this process proceeds. We believe, however, that optimal mechanisms have yet to be established to provide for constructive interaction in this process among the public, policymakers, and the health sciences community. This situation is not surprising, given the lack of precedent for enlisting public participation in such a planning process.

The deadline of Fall 1979 for the completion of the plan is unrealistic, particularly in view of the time that has already been consumed in the relatively non-controversial task of stating broad principles. To develop a first rate plan that attends to the many complex issues in health sciences research, and to do so in a manner that continues to provide for adequate review and commentary by the interested parties at each stage of the plan's development, requires more than the several months remaining until the Fall deadline. Even in the best of circumstances, in which a planning process is well defined and the information needed is readily at hand, the time available might be too short. Since the translation of these broad principles into a detailed plan and budget may shape health sciences policy for many years to come, we urge that this process be afforded sufficient time to help assure the development of a comprehensive plan of high quality.

We also urge that this planning effort lead to the development of a permanent planning mechanism as an important instrument for the determination of national health science policy. Such a mechanism will be needed at a minimum for the regular review and revision of this current plan in an orderly, efficient way. More generally, a permanent capability for long-term planning will provide a view of research support that extends beyond yearly budget cycles or current political pressures, and will establish a clear locus for the needed consideration over time of such issues as the appropriate balance of fundamental and applied research, the relationship of the federal government and non-governmental research institutions, the role of research in regulatory agencies, mechanisms for coordinating health research throughout DHEW, approaches to establishing priorities within applied research, and the many other policy problems noted throughout this Report. The character and composition of this planning function need very careful thought, particularly with regard to the relationship of the planning mechanism not only to the health-related agencies of DHEW, but also to other federal agencies engaged in health research, and to the Congress, the Office of Management and Budget, and the Office of Science and Technology Policy.

PART I

THE RESEARCH ENTERPRISE FOR HEALTH

In commenting on DHEW's plans to develop a five-year strategy for its support of health research, the Committee has considered the past accomplishments and future promise of the health sciences enterprise. We have drawn on the record of congressional inquiries into publicly supported health research, documents such as The Report of the President's Biomedical Research Panel, specific assessments of the state of selected areas of health research, and other studies of the nation's commitment to the health sciences. The picture that emerges from these multiple sources is one of progress, hope, and firm public support for a vigorous national program of research directed towards improving health. Such support is focused on a set of important and enduring goals.

These are to:

- advance the fundamental knowledge base of the health sciences;
- translate fundamental knowledge into improved diagnostic, treatment and preventive interventions, and thereby help to alleviate suffering, improve the quality of life, and enhance survival;
- provide the basis for regulatory actions designed to promote safety and health; and
- provide the basis for informed decision making on health policy matters, including the organization, delivery and financing of health care.

Accomplishments of Health Research

Research and development in the health sciences have resulted in important contributions to the health of the public. Some of the advances that illustrate these contributions include:

- the development of antibiotics and other drugs for the control or cure of many bacterial and parasitic diseases;
- the development of immunizations for a variety of viral and other infectious diseases;
- improvements in surgery and anesthesiology which have afforded effective surgical treatment for a wide range of disorders such as congenital anomalies, many forms of neoplastic growth, valvular heart disease, and skeletal disorders;

- the ability to eliminate many gross nutritional deficiency diseases;

- deepened understanding of reproductive biology, leading to the development of contraceptives and treatments for infertility, both of which hold out the promise of voluntary reproduction;

- the development of effective noninvasive diagnostic procedures such as ultrasound imaging and radionuclide techniques;

- the construction of a wide variety of prostheses including joints, heart valves, and pacemakers;

- the development of effective pharmacologic agents to manage such serious disorders as cardiac arrhythmias, angina pectoris, congestive heart failure, hypertension, Parkinson's disease, psychoses and several neoplastic diseases;

- major advances in prenatal diagnosis and neonatal therapy to ease the burden of certain congenital disorders; and

- the treatment of end-stage kidney disease through dialysis and transplantation.

Factors Related to Health and Disease

A parallel advance has been a broadening and deepening in our understanding of the many factors related to health and disease. In recent years, the roles of personal habits and environmental factors in health have become more fully appreciated. Individual behavior--smoking is perhaps the clearest example--is now recognized more widely than ever as an important determinant of health. There is also growing appreciation of the impact on health of changes in the physical and chemical characteristics of the human enviornment. Particularly in recent decades, we have been exposed to a range of new substances that were unknown to our ancestors, especially pollutants in the air, ground, and water. Even the nature of stress in our society, and our personal relationships at work and at home have changed. The effects of these many factors and changes are profound, some are probably as yet unrecognized, and most of the long-term consequences are poorly understood. Finally, the organization of health care services--their cost, accessibility, continuity and efficiency--also has a major influence on health status. Health care services mean little if they are not applied appropriately to individuals

and populations.

Challenges for Health Research

Important and difficult problems remain, despite these numerous advances in our understanding of basic life processes and the many factors influencing health and disease. Causes of death and disability have changed in recent years, producing a new profile of the burden of illness that is enormously challenging to our ability to achieve effective disease prevention and treatment. The success of public health workers and biomedical scientists in combatting infectious diseases has shifted our orientation to accidents and chronic illnesses as the major limitations on life and health. Accidents now rank as the number one cause of death among all persons in the age range of 1 to 38 years. Cardiovascular disease and cancer are leading causes of death in older people. Mental disorders including the schizophrenias, depressions, and senile dementia and the problems associated with alcohol and other drug abuse are widely prevalent and exceedingly costly to society. Diabetes, chronic kidney diseases, and arthritis have become major sources of costly and often painful disability. Dental diseases remain a heavy health burden.

Problems relating to the organization and financing of health care also are of growing concern, and have been intensified by the recent large increases in health care costs. The rate of increase in these expenditures, especially in hospitals, is growing much more rapidly than the general rate of inflation. Yet effective means have not been found to contain these costs, while still protecting the health of the public and providing needed care. There is reason for concern that some population groups—particularly the totally disabled, those with low incomes, and the socially deprived—do not have adequate access to health care because of financial, and geographical barriers. Others may find the cost of adequate care a threat to financial security. It is also apparent that certain age groups, such as the elderly and the adolescent, often obtain inadequate and inappropriate health care.

Another serious problem involves the technology of medicine--the operations, diagnostic procedures, and equipment that are at the heart of much of medical practice. Despite professional standards that emphasize quality of care, the rapid pace of invention and development in recent decades has outrun the evaluation of new medical practices. Research is needed not only on the effectiveness and safety of these many interventions, but also on the actual methods for measuring the costs, risks, and benefits of medical procedures.

There is also concern that the extent of public understanding of vital health matters is inadequate, as suggested, for example, by the nation's relatively low rate of polio immunization, the continued prevalence of smoking, and nutrition and exercise habits that are unhealthy. It is also clear that present health education strategies are not adequate to bring about needed changes in the health-related behavior of our citizens.

The Range of the Health Sciences

An effective response to these widely varied challenges to life and health requires a vigorous program of research across the full range of the health sciences. A partial list of these intertwined and complementary disciplines follows. It is important to emphasize that many of these sciences include both fundamental and applied components:

- the biomedical sciences, which inquire into the basic nature of life and hold out the ultimate promise of disease prevention and improved care through deeper understanding of life processes;

- the clinical sciences, which bridge fundamental research and medical practice, and provide the links between health problems and the investigation of those problems at the most basic level;

- the population-based sciences such as epidemiology and biostatistics, which examine the distribution, determinants, and effects of disease, and the effectiveness of preventive methods and therapies;

- the behavioral and social sciences, which study the interaction of cognition, emotion, and bodily response; individual motivation, voluntary control, and modification of behavior; learning and decision making; and organizational structures and processes;

- biophysics, bioengineering and clinically-oriented medical engineering and medical physics, which provide conceptual and analytic strengths that advance our understanding of fundamental biological and medical processes, and make it possible to achieve successful and practical solutions to difficult problems in clinical medicine;

- hybrid sciences, such as the nutritional and environmental sciences, which draw on basic and applied disciplines in a multidisciplinary approach to their respective problems;

- health services research, which studies the health care system--its structure, processes and effects--and looks at such practical problems as the organization, quality, accessibility, delivery, and cost of personal health services;

- technology transfer, which is concerned with the study of the sources of innovation; the roles of the private and public sectors in the generation and application of new health technology; the economic, political, and regulatory influences that promote or impede the development of prototype technology, which is an intermediate stage between applied research and large-scale production; and the development of sound methods for the evaluation of existing technology and for predicting and evaluating the impact of new technology.

Each component of these health sciences has special contributions to make to the social goal of improved health and disease prevention. An overriding emphasis on any one part of the spectrum, or the exclusion or neglect of individual fields, would limit the benefits to be derived from scientific research. Even a brief review of the Interdisciplinary Cluster Reports of the Report of the President's Biomedical Research Panel reveals that promising advances are occurring in many areas of the health sciences, with both near-term and long-term payoffs expected for improved health status.

In short:

- The biomedical and clinical sciences have made extraordinary contributions to the quality of life and health in recent decades.

- A new profile of illness, which includes much behavior-related and chronic disease, now burdens the nation.

- We have grown in our understanding that many social, behavioral and environmental factors, including the organization of health care services, strongly influence health and disease; and we have begun to strengthen the science base to study these factors.

- It has become apparent that research advances in all components of the health sciences are needed to realize additional gains in health status.

The Support of Health Research

The goal of effective treatment and prevention of disease cannot be reached without the continued nurture and support of health research. This goal also cannot be achieved by crash programs nor can it be attained within a fixed schedule of years. The quest will not be productive if it is turned on and off arbitrarily, nor can it succeed if held at a standstill without opportunity for the growth necessary to develop new fields of study, to pursue new opportunities in existing fields, and to support, maintain, and renew research institutions. What is needed is decades of steady, hard work by skilled and imaginative investigators giving continuity to the research activity. This undertaking must be vigorously supported to insure that talented investigators will have the funds and facilities that are essential to the achievement of this goal.

The Committee realizes that such a program may be difficult to implement in an atmosphere of budgetary restraint. Health research, however, constitutes a very small part of health expenditures, and is the part most likely to enhance the quality of life and to lead to cost savings over the long-term by discovering improved ways of treating and preventing disabling and economically burdensome diseases. Moreover, there is ample evidence that research directed toward improving health is appreciated by the American people and has their strong support.

The Committee believes that resources now devoted to the health sciences are seriously inadequate in relation both to the need for the results of health research, and to the scientific opportunities in these fields. Health care should be based soundly in science. Yet in 1977, only about 3.4 percent of total health expenditures was devoted to health research*--a figure that, we are convinced, it insufficient and should be increased. We recognize that there is no simple answer to the question "how much is enough?" We believe, however, that with thoughtful study, it should be possible to determine a level of support for the health sciences that is both realistic in terms of the national economy, and sufficient to foster an enriched and highly productive national health research enterprise. Such policy analysis should be a significant element in the work of a permanent planning mechanism which we urge grow out of this current planning activity, as suggested below (see 22 and 23).

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*Source: "Basic Data Relating to the NIH, 1978." Total national (public and private) health expenditures in 1977 were $162.3 billion; total health R&D (public and private) was $5.5 billion; total federal health R&D was $3.3 billion.

In taking the position that current resources are insufficient, the Committee recognizes that it could be charged with promoting a self-serving interest. We believe nonetheless that health sciences research merits increased support due to its high social priority, which in turn is derived from its demonstrated productivity leading to extraordinary improvements in life and health, its long-term promise for diminishing the economic burdens of illness, and its enhancement of the quality and duration of life for our people. In advocating increased support, we also believe it is the responsibility of the health sciences community to assure that funds are spent efficiently and prudently, through careful research administration and such mechanisms as the peer review system, which operates to insure that only scientific projects and investigators who meet very high standards are supported.

The expanding opportunities in the health sciences, the current burden of illness, our growing appreciation of the many factors that affect health, and the strong support of the public for the social priority of research to improve health add further strength to our view that policies should be formulated which increase and make predictable the support for such work. The Committee therefore wishes to cite with approval the statement of the Panel on Health Regulation and Promotion, of the National Conference on Health Research Principles:

> We do not accept the premise that resource limitations preclude significant expansion of the health research budget. That is a political judgment, not a reflection of economic necessity... [Current research expenditures are] a miniscule investment in relation to the size of the enterprise whose effectiveness rests upon it. Health research, basic, applied and related to the efficacy of preventive, therapeutic and regulatory services, provides major hope of improvements in the public health. It is penny wise and pound foolish to economize on the investment in obtaining the knowledge which can permit effective action for better health.

PART II

THE CURRENT PLANNING ACTIVITY FOR HEALTH RESEARCH

The Values of Planning

This Committee endorses the initiative of Secretary Califano and the DHEW leadership in directing that a multi-year strategy for health research be developed. In this effort, DHEW is being highly responsive to a number of factors affecting health research: widespread recognition that some areas of research have not been and are not being supported at a level commensurate with the need for results from these lines of inquiry; deep concern that the nation's commitment to fundamental research not waver; an appreciation that abrupt changes in research emphases at the federal level can cause wasteful dislocations in scientific programs; a recognition that funding priorities within applied health research do not necessarily reflect either the nation's profile of illness or scientific opportunity; and a general concern about the future of federal support for health research in an atmosphere of intense fiscal restraint. Given these many factors, long-term planning for health research is desirable and should be useful.

A well conceived plan should consider: the extent and distribution of current research efforts; the nation's burden of illness; the disciplines that show special promise for advancing the health of the public; problems inadequately studied; excessive duplication of activity; and areas where personnel and facilities must be developed further or better utilized. With such information in hand, it should be possible to address the perennial and difficult problem of establishing research priorities in an informed and constructive manner.

A well-designed health research plan should benefit the individuals and agencies involved in its preparation by fostering consideration in a deliberate and thoughtful manner of their roles and functions in health research. The missions of the individual agencies should become more crisply defined and areas of overlap and neglect more visible. The potential for increased interdisciplinary, multidisciplinary, and interagency research should become more apparent when all health research is arrayed in one plan at one time.

A comprehensive plan should also promote increase predictability and stability in federal support for health research--a goal which we strongly endorse. Federal funding of health research has often been erratic as a result of shifting priorities and budget levels, and of changing leadership, among other factors. Even outstanding scientists, in many areas of study, have suffered from the disruptions of fluctuating funding. The trend to shorter federal research funding cycles for centers, programs, and projects enhances the uncertainties and changeable character of research support. A hoped-for consequence of planning is increased predictability in funding and the possibility of longer-term funding, both of which would promote sustained attention to health needs. Research, by its very nature, is a long-term process that flourishes in a stable environment, particularly because scientific results are obtained at unexpected times and often through unanticipated channels.

Developing a research plan provides excellent opportunities to make information about health problems widely available and to foster communication among the public, its elected representatives, and the health sciences community about the nature, scope and goals of health research. The taxpayers who pay for research deserve to be provided with evidence that the research community is aware of the nation's health needs, and that it is pursuing new knowledge pertinent to these needs with vigor and excellence. Even though the results of research cannot be planned, the public has a right to know and debate broad priorities and emphases.

A comprehensive plan should also create an awareness of the role of the private sector in scientific inquiry. It could enhance the possibilities of integrating the efforts of the public and private sectors, so as to use existing resources more effectively, and to share the results of research in both sectors cooperatively and efficiently.

The Difficulties of Planning

The Committee also wishes to point out some difficulties associated with research planning generally and with this activity in particular. For example, planning may lead to inflexibility in research priorities, and may limit the ability of the government to adjust its research agenda to new social needs or scientific leads. We strongly urge, therefore, that the research plan eventually developed be reviewed and revised often in order to avoid the inefficiencies and missed opportunities that derive from inflexibility.

The great diversity and complexity in the many fields of study that comprise health research may be overlooked in the effort to develop a single plan for all of health-related research. Different types of planning processes, at different levels of specificity, are required for the various components of health research. For example, the essential value of fundamental research is to provide the advances in knowledge which form the science base for future progress. Major fundamental discoveries may change a field quite suddenly—a situation which demands flexibility in directions and resource allocations. As such, fundamental research can be "planned" only at a general level. In some areas, such as the environmental sciences, aspects of planning that encourage an interdisciplinary or multidisciplinary approach to problems are highly desirable. And in some areas, such as the last phase of developing a new drug, device, or technique, where the knowledge base is generally adequate, yet another type of planning which is more detailed and goal-specific may be appropriate. All components of health research are not alike, and therefore planning in each area may require different levels of specificity.

Another, more philosophical concern is that in developing a plan for health research, it is possible to gloss over the overwhelming extent of our ignorance about life, human behavior, social organization, and disease processes. Knowledge is lacking in many areas of health and behavior, such as the mechanisms of the aging process, the biochemistry of alcohol addiction, the role of our new chemical environment in cancer, the relationship of diet to health, the role of exercise in cardiovascular disease, effective techniques for health education, and the cost-effectiveness of many new technologies—the list is very long. It is important that in the effort to set research priorities, and to bring the sense of order to research that planning suggests, the great need for new knowledge about an almost infinite number of phenomena be kept clearly in mind.

An additional concern is that a plan may set up false expectations that the processes and results of research can be planned and produced "on schedule." While this may be true in some highly applied areas of study where a strong base of fundamental knowledge exists, many of the results of research appear in unpredictable and unexpected ways, and cannot be anticipated in an orderly sequence. Thus, planning for health research bears the

risk of erroneously suggesting to policymakers and other interested parties outside of the scientific community that desired goals (such as "a cure for arthritis") can be attained at specified times through careful planning. Needs for health research can be identified, broad research strategies can be planned, and crude estimates of scientific personnel needed in the future can be made. But the tangible results of scientific inquiry cannot always be defined or planned in advance. The more fundamental the science, the truer this statement is.

Implementation of a long-term federal research plan is, of course, hampered by the nature of governmental processes. The Congress appropriates money for health research yearly, not in five or ten-year blocks. Annual budgeting means that each year the federal approach to health research is reexamined, and often changed, sometimes only minimally and sometimes quite profoundly. The rapid turn-over of personnel in Congress and the Executive Branch also means that long-range plans are reviewed by an ever-changing group of policymakers, each of whom may wish to give a different emphasis to the forward plan.

There are also risks in planning health research that are unique to the present time and place and that may have serious future consequences. The current atmosphere of fiscal restraint may lead to a plan whose primary effect is to reduce funding for health research in the immediate future. It must be remembered that this plan is intended to guide health research from about 1982 to 1987--not this year or next. Thus, careful attention must be given to avoid having current fiscal constraints prejudice the future of support for health research.

A Permanent Planning Mechanism

Despite these numerous and generally obvious limitations on research planning, we reiterate that a multi-year plan for health research remains a desirable activity. We urge, however, that this planning effort lead to the development of a permanent planning mechanism for the health sciences as an important instrument for the determination of national health science policy. Such a mechanism will be needed at a minimum for the regular review and revision of this current plan in an orderly, efficient way. More generally, a permanent capability for long-term planning will provide a view of research support that extends beyond yearly budget cycles or current policital pressures and will establish a clear locus for the needed consideration over time of such issues as the appropriate balance of fundamental and applied research, the relationship of the federal government and non-governmental research institutions, the role of research in regulatory agencies, mechanisms for coordinating health research throughout DHEW, approaches to establishing priorities within applied research and the many other

policy problems noted throughout this Report. The character and composition of this planning function need very careful thought, particularly with regard to the relationship of the planning mechanism not only to the health-related agencies of DHEW, but also to other federal agencies engaged in health research, and to the Congress, the Office of Management and Budget, and the Office of Science and Technology Policy.

The Process to Develop the Current Plan

The Committee wishes to make a few observations on specific aspects of the process being used to develop the requested plan.

- We support DHEW's commitment to public participation and open processes in the development of this plan, and encourage DHEW to assure that at each stage of the plan's development, opportunities be provided for commentary from many sectors. This includes review when the plan is implemented as well as in these planning stages. All parties need to give thought to finding ways of structuring this public discussion so that it is well informed and constructive.

- The October 1978 Conference on Research Planning Principles was commendable as an experimental effort to involve the public in research planning. Although conference participants were afforded a limited opportunity to express their views, we believe that the conference structure and time constraints did not allow for the useful interactions among the public, the scientists, professional societies, and others that the conference sponsors had intended.

- The preliminary, relatively easy aspects of research planning--namely, the development of general principles underlying health research--have taken many months. We agree that a statement of broad principles is an important first step. However, if the deadline for the completion of this plan remains the autumn of this year, the risk grows daily that too little time will remain for the more difficult and necessarily controversial task of establishing research priorities and sorting out agency missions to pursue these priorities. There are now only seven or eight months remaining until the autumn deadline--an interval that is inadequate for constructing a first rate research strategy, even if a process were already in place to reach this goal. In the present atmosphere of less than complete agreement on these principles, the purposes and scope of the plan, and even the process to produce the plan, the existing deadline becomes even more unrealistic.

- We suggest further that many important issues have not yet surfaced in this process or have been inadequately explored in the principles. Section IV of this Report highlights some of these issues.

- We are also concerned that if the most difficult questions are not addressed until late in the process, there will not be sufficient time for the public, the health research community, and other interested parties to discuss suggested policies and priorities thoughtfully before the plan is issued in final form. The first phase of developing the principles attempted to include such discussion, but the need for detailed review will be much more pressing in the later phases of the plan's development, when the numbers and priorities are on paper for all to see.

- By confining the scope of the plan to only those agencies within DHEW that support health research, important components of the total federal research effort pertinent to health are being omitted. The Veterans Administration for exmaple, supported close to $112 million of health research in 1977, principally through the Veterans Hospital system. The Departments of Defense and Energy also are involved in health research. During 1977, the Department of Defense spent $124 million on health research, and the Department of Energy, $203 million. Similarly, the National Science Foundation reported $58 million of such research in 1977. The value of the DHEW plan would be enhanced if it were developed in relationship to the research activities and needs of these other agencies.

- We suggest that the long range utility of the plan will be enhanced if key leaders from Congress, the Office of Management and Budget, and the Office of Science and Technology Policy are kept apprised of the plan as it is developed.

PART III

THE RESEARCH PLANNING PRINCIPLES

General Commentary

The Committee has several general comments about the principles:

A. The major message of the principles and of the October 1978 Conference during which they were discussed is a vigorous reaffirmation of existing federal support for the health sciences, and a clear call for an increased commitment to new and neglected areas. In neither the principles nor the conference was there a suggestion of rigidly limiting or reducing federal support of health research. We discern no themes in the principles or conference that suggest a major overhaul of the nation's health research commitment, except, as mentioned, to increase support for selected areas.

B. A key feature of the principles is that they embrace a concept of the health sciences that is much broader than was the view as recently as ten years ago. Rather than equating health research with biomedical research only, the principles recognize the importance of such areas a epidemiology and biostatistics, environmental sciences, health services research, and the behavioral and social sciences for disease prevention and health maintenance; the Committee would add to this list biophysics and bioengineering, and clinically-oriented medical engineering and medical physics. The Committee regards this broadened concept as a desirable advance in policy formulation, and supports efforts to translate this perspective into tangible and adequate support for the full complement of the health sciences.

C. The Committee also strongly supports the view expressed in various subsections of the principles—and throughout the conference report as well--that fundamental science is the cornerstone of health research, and that support for new and neglected areas of health science must not erode the core support of fundamental research.

D. Throughout the principles and the conference discussions as well, the value of excellence in all areas of health research has been mentioned repeatedly. This Committee concurs that a commitment to excellence should be the prime determinant of support for health research. The public support of the health sciences enterprise demands that excellence be the ultimate criterion by which research allocations are made. Poor quality research is not worth doing, no matter how pressing the problem to which it is directed. The peer review system is a principal mechanism for assuring such excellence and should be applied as widely as possible to health research funding.

E. Because of the generality and occasional ambiguity of the principles, we believe that they will be of only modest help in establishing detailed priorities and budgets and in sorting out the research missions of DHEW agencies. The difficult decisions needed to develop the actual research plan still lie ahead. We do not know what mechanisms will be used to translate these necessarily broad principles into a useful forward plan, or which set of decision-makers will actually set the five-year research priorities for DHEW. In the absence of such knowledge, we urge again that there be adequate opportunity for review and revision of the substantive decisions yet to be made in the light of these general principles.

The Five Sets of Principles

In the five sections below, the draft DHEW principles themselves are reproduced at the beginning of each section and enclosed in quotation marks. They have been numbered to assist in our review. After each set are presented the Committee's commentary and, where appropriate, specific discussion of individual principles. Differences in the nature of the commentaries on the five sets of principles and some overlap in the discussions are to be expected given the variability in style and some redundancy in the principles themselves; furthermore, this Committee was itself divided into five panels to review the principles, as listed as the beginning of this Report.

A. Principles Regarding Fundamental Research

"This focus addresses the need for fundamental research (the search for knowledge about fundamental processes of biology and behavior), the creative process that governs this type of research activity, and the role of fundamental research in improving public health and providing the essential knowledge base upon which other Department health missions rely. This focus also encompasses the issue of stability of research support and the relationship of fundamental research to other activities in the health research continuum.

A1. Principle--A national commitment to fundamental research is essential to meet the full range of public health expectations.

A2. The Federal investment in fundamental research must be maintained to develop the knowledge base essential to support the health research continuum that extends through applied research to the prevention, diagnosis, and treatment of disorders and diseases and to rehabilitation. This should be recognized as a long-term investment that provides the freedom for scientists to pursue diverse research topics that may not be immediately relevant to practical health problems.

A3. This national commitment must include long-term, stable support for fundamental research. This support is essential for the maintenance and strengthening of the scientific knowledge base related to all DHEW health missions.

A4. The development of new knowledge through fundamental research, and its application, has a significant impact in improving our ability to prevent disease and the effectiveness of treatment and rehabilitation.

A5. The decision to fund fundamental research in a given area should be based upon an assessment of related ongoing research and new scientific opportunity. The peer review system must be regarded as an efficient and essential instrument in the conduct of research grant programs.

A6. Investigator-initiated research proposals must continue to be emphasized in the conduct of fundamental research, and must be restored to their previous prominence as the primary mechanism in the allocation of funds.

A7. Recognition must be given to a broadened concept of health factors and a research base developed to investigate them. This base should include studies in the behavorial sciences and population sciences such as epidemiology and biostatistics.

A8. The Directors of NIH and ADAMHA should conduct annual program reviews to update projections and review amounts needed in fundamental research, to identify new areas warranting support, to curtail inappropriate distribution among areas already being supported, to monitor the allocation of resources to fundamental research, and to review the allocation of resources among the various fields."

Commentary

Central to any discussion of fundamental research is the issue of what is to be included in such research. We find the DHEW definition adequate,* particularly because it includes in the last phrase research which grows out of clinical problems, even though the goal of such inquiry may not be immediately applicable to diagnosis, treatment, or prevention of disease. We endorse a major theme of all the principles, that fundamental research is a component not only of the biomedical sciences, but also of the behavioral and social sciences, and population sciences such as biostatistics and epidemiology. We also suggest that fundamental research in these areas has not been adequately developed.

As we note at the beginning of Part III, we endorse the broadened definition of the health sciences, but emphasize here again that support for fundamental science must not be lessened in the effort to increase funds for new and neglected areas. Any dilution or further diminution in support of fundamental biomedical research in particular would be in direct conflict with the message of principle A1, which we find incontestable. Fundamental research, often without reference to, or motivation by, a specific health problem, has contributed the knowledge needed for major health advances. The Comroe-Dripps analysis** shows conclusively that more than forty percent of the crucial and decisive developments that underlie present capabilities in cardiovascular and pulmonary medicine and surgery derive from untargeted, fundamental research in the biomedical sciences. We believe that the germinal role of fundamental research would be similarly illuminated were comparable studies made of successful advances in other medical specialties. There is widespread agreement that long-term solutions to major health problems will not be forthcoming without

*"Fundamental Research - a search for new knowledge directed towards increasing our understanding of life, health and disease. The terms of reference are scientific inquiry into fundamental processes of biology and behavior. This science base includes both undifferentiated research tending to be isolated from a specific disease orientation, and clinically-oriented research focusing more on specific disease processes."

**Comroe, J.H., and R.D. Dripps. "The Top Ten Clinical Advances in Cardiovascular-Pulmonary Medicine and Surgery Between 1945 and 1975: How They Came About." Final Report, January 31, 1977. Supported in part by Contract 1-HO-1-2327 from the National Heart, Lung and Blood Institute, and grants from The Commonwealth Fund and The Burroughs Wellcome Fund.

a more detailed knowledge of the origins and mechanisms of disease, and the nature of human behavior and social interaction. Society's "burden of illness" is in fact a burden of ignorance. There is no alternative to fundamental research for eliminating that ignorance. Fundamental research is, indeed, the lifeline of medicine.

Principles A2-A5 emphasize that fundamental research is essential and must be fostered to support the entire range of health research that is encompassed by all DHEW health missions. As general statements they are laudable and should be affirmed. But the intent and impact of these statements, particularly as they bear on the specific budgetary decisions yet to come, are obscure. These statements could be signaling a commitment to develop or foster new, improved mechanisms for utilizing basic research advances in all areas of DHEW's concerns. We would support such a commitment. An additional or alternative interpretation is that fundamental research should become more "targeted" to DHEW health missions, e.g., prevention, diagnosis and treatment of disease, rehabilitation, regulation, health education, and the like. Such targeting would misrepresent the essential nature of fundamental research, which is that it is not directed towards specific diseases or social problems. We support the notion that the results of fundamental inquiry are necessary to meet the multiple missions of DHEW, but not that such basic research itself should be more directed or mission-oriented.

During the October conference, and occasionally in the language of the principles themselves, the notion is raised of diffusing responsibility for administrative control of fundamental research to other agencies of DHEW beyond NIH and, to a much lesser extent, ADAMHA. As regards basic biomedical research only, we would not support such a diffusion. Were many other agencies to become involved in basic biomedical research, there would be an increasing likelihood and perhaps even inevitability that such research would be targeted to the sponsoring agencies' missions rather than remaining truly fundamental and therefore not mission-oriented. Furthermore, inevitable duplication of work, and the necessity for establishing a separate (though parallel) peer review system to assure high quality research would be wasteful.

With regard to the focus of administrative responsibility for fundamental research in such fields as the behavioral and social sciences, epidemiology, and biostatistics, the DHEW principles are not explicit. We recognize that a number of DHEW agencies beyond NIH and ADAMHA may wish to pursue such fundamental research in an effort to meet the long-term needs of their specific missions. The Committee believes that this important and difficult issue deserves much careful study so that the eventual decisisions concerning the administrative loci for fundamental research in these areas will serve to assure excellence in research and high quality in peer review, and will minimize redundancy.

Principles A2 and A3 identify the costs of fundamental research as a "long-term investment" and argue that the nation must commit "long-term stable support" to that activity. Though commendable, these phrases are too vague to be evaluated in practical terms. Does this willingness to view fundamental research as a long-term investment take account of the budgetary requirements for:

a. Training future generations of scientists, technicians and teachers; upgrading obsolete, inadequate laboratory/clinical facilities and equipment; providing the essential experimental animal facilities?*

b. Assuring the health, integrity, and vigor of the universities and institutions in which the bulk of high quality fundamental research is performed?*

c. Developing novel, imaginative mechanisms that would provide secure funding to meritorious, promising programs, to investigators of proven ability and accomplishment, and in a fashion that would also encourage and support the growing body of talented young scientists?

d. Reducing the uncertainties inherent in present research funding patterns and eliminating the consequent starts, stops, and delays in research programs that are wasteful of research effort, time, and money?

e. Relaxing the requirement for frequent renewals of research support for the most productive and successful scientists, while still retaining the capability of monitoring the quality of ongoing research and of evaluating new leads, challenges, and initiatives?

f. Expanding sources of support, such as the Biomedical Research Support Grant (BRSG), that improve the capability of institutions and investigators to capitalize on new research opportunities and to transcend disciplinary barriers?

We urge that the planning activity take full note of these many issues when trying to implement the stated commitment to long-term, predictable funding.

Principles A6 and A7 discuss the problem of identifying the most promising investigators for research support and of ensuring that such research is of the highest quality and potential. We strongly support

*See also the comments regarding the principles on Research Capability

the view that investigator-initiated research proposals should be the primary mechanism for allocation of funds in fundamental research programs. The statement of the October Conference Panel on Fundamental Research, "This mechanism allows for the maximum expression of individual ingenuity and competence in the formulation of research strategies, and therefore ensures a freshness and diversity of approach that would be difficult to evoke by any other means," emphasizes the same preference. But there is also merit to considering alternative modes of investigator-initiated proposals for core grants, program projects, interdisciplinary programs, etc. These should be implemented when there is clear and convincing evidence that such mechanisms create opportunities, capabilities, and facilities that are not attained as well through competitively awarded grants to individual investigators. This is especially important where it is desirable to pool the strengths of various disciplines, as in fostering the vital interplay of the physical sciences, engineering, and the behavioral and social sciences with the biomedical sciences.

We also support strongly the October Conference Panel's conclusion that the peer review system is the most effective, cost-efficient administrative procedure for assessing the quality, opportunities and directions of the nation's fundamental research programs. Further efforts are needed to improve the peer review system throughout DHEW for both basic and applied research. Within the NIH in particular, the peer review system needs to be strengthened so that its evaluations are not compromised by unreasonable work loads or by marked lack of uniformity in priority scores and funding across the multiple institutes. We also suggest that while consideration of national need will inevitably affect the priorities of different research activities, diligence is required to guard against the support of low quality even when such research is apparently responsive to these needs.

Principle A8 calls for annual program reviews "to update projections and review amounts needed in fundamental research, to identify new areas warranting support, to curtail inappropriate distribution among areas already being supported, to monitor the allocation of resources to fundamental research, and to review the allocation of resources among the various fields." It is difficult to imagine how enterprises such as NIH and ADAMHA, overseeing hugh expenditures and large numbers of scientists and facilities, could function effectively without such periodic review, and indeed considerable review and planning are currently performed already. In this regard we urge that the recommendation made by the October Conference Panel, that the NIH and ADAMHA Directors consult broadly with outside scientific bodies in making their assessments of ongoing or prospective research programs, be given serious consideration. An additional recommendation which we support is that the Directors of NIH and ADAMHA have sufficient discretionary funds to encourage and promote new areas of fundamental research e.g., convening conferences to stimulate

interest in new areas or the modest use of special funding mechanisms to catalyze direct involvement of investigators in a new field.

A word of caution concerning Principle A8 is appropriate: there is a possibility that annual reviews, curtailments, and re-distributions of resources could jeopardize or compromise the previously stated commitment to long-term, predictable support for investigators and research programs. We recognize that both goals are important; careful attention and creative administrative mechanisms are needed to balance the two. For example, rather than making abrupt changes when undertaking needed program redirections, and thereby violating the need for predictability and continuity, such changes should be phased in gradually.

In the context of the present commentary, it is important to raise an issue not addressed directly in any of the principles, but which nevertheless has substantial impact on the conduct of health sciences research. We are increasingly concerned about the escalating costs in time and money attendant upon compliance with the growing body of federal, state, and local regulations governing the conduct of research. Experimentation with human subjects and even with materials from human sources is at times unnecessarily burdensome. Similarly, excessive bureaucratic restrictions could impede recombinant DNA research and retard the advances that this powerful new methodology promises. We recognize that there are benefits as well as costs in regulatory processes related to research. The goals of protection of human subjects, and accountability in the uses of public funds are of great significance and value. Nonetheless, the costs of regulation to research have to be in reasonable balance with the benefits achieved. We suggest that an analysis be undertaken of the role of regulation in the health sciences to examine such issues as:

- the risks in research towards which regulation is directed--both the risks to subjects and to investigators, and the liability of investigators and institutions for injuries resulting from experimental procedures themselves or from the products and devices derived from such research;

- the various mechanisms available for handling such risks;

- the possibility that the aggregate effect of individual regulations may inhibit needed and valuable research; and

- the overall benefits and costs of regulation.

Such an examination should include the views of the public, those who conduct and participate in research, and those involved in its regulation.

B. Principles Regarding Clinical Applications and Health Services Research

"This focus addresses the development of the knowledge base to support the health care function, the need to acquire, validate and apply new knowledge, the characteristics of and requirements for applied, problem-oriented research, and the need to retain and strengthen the Department's pluralistic approach to the support of applied research.

B1. Principle--To improve the quality of health care, prevent disease and contain health care costs, the health care system requires, in addition to new knowledge developed at the fundamental level, the support of applied, problem-oriented health research.

B2. The development of an adequate knowledge base for the fulfillment of health care missions must be supported. This includes strengthening fundamental research, as well as applied research approaches to the solution of health problems, in order to develop systematic ways to meet the needs of non-research health agencies for specific knowledge. This includes giving an appropriate priority for stable funding to certain research areas, such as epidemiology, biostatistics, toxicology and nutrition.

B3. The health research agencies must be responsible for assisting the health regulatory, care and policy-making agencies, whenever a formal request for assistance is made which clearly falls within the mission of the research agencies. Consideration should be given to the resources necessary to comply with such requests. The many applied health research needs which cannot be filled through such a system should be addressed by applied, mission-related research funded through the budgets of the health regulatory, care and policy-making agencies. Coordination and dissemination of the required knowledge should be provided through interagency committees responsible to the Office of the Secretary.

B4. The validation of preventive and therapeutic measures before their introduction into the health care system must be assured and standards for their application in medical practice determined. This includes the validation of new regimens in terms of their efficacy, safety, and cost-effectiveness.

B5. After the introduction of preventive, therapeutic and diagnostic measures into actual use, monitoring programs should be employed to evaluate both their costs and benefits. This function should be part of a strengthened activity in health services research and technology assessment within the Office of the Assistant Secretary for Health. In addition, a fixed and adequate percentage of the budget of health care agencies should be devoted to these monitoring programs.

B6. The conduct of fundamental and applied research to improve methodologies for statistical, epidemiological, sociological and economic measurements in the health fields must be supported. This includes a long-term commitment to continuation and expansion of applied measurement research in statistical and survey methodology to provide improved statistics on morbidity and mortality, disease prevalence and incidence, disability prevention, behavior, enhancement of quality of life outcomes, the organization, delivery and financing of health care services, and new methods to facilitate epidemiologic analyses of large health data files. Funding for such methodologic research should occur at a reasonable level independent of support for specific health programs.

B7. A strategy must be adopted for funding health research which includes provision for specific program expansions for the support of clinical applications, clinical trials and health services research activities without diversions of funds from fundamental research. This includes the development and funding of multi-year, high-cost investigations and clinical trials as direct add-ons to specific agency programs where an adequate and available science base exists. If budgetary increases cannot be accomplished to provide such funds, then the health care dollar should be considered as a source. Priorities should be established for funding these activities, based on the state of the science base related to each effort, the feasibility of each effort, based on the state of the knowledge and available resources, the potential impact of the trial on research, health care, the burden of illness, and various ethical considerations. The public sector, academic centers and appropriate elements of the private sector should be an integral part of this process.

B8. The scientific merit of proposals for clinical investigations, clinical applications and health services research is a necessary condition for support. Each agency sponsoring such research should administer a peer review process to determine the scientific merit of each research proposal, and the reviewers should include nongovernment persons from appropriate disciplines.

B9. The systematic dissemination of health services research results to providers of health services requires more emphasis, so that advances in knowledge are applied by the health care system as promptly and effectively as possible. However, care must be taken to avoid premature introduction of unproven new health technologies. Further research and evaluation are needed to determine the most efficient and effective methods of technology transfer.

B10. Consensus development approaches for providing information and technical transfer should be strengthened to assure the involvement of all constituencies relevant to the specific health technologies addressed.

B11. Priorities for health services research support must be defined, and the social value of anticipated findings should be a determining factor in this process. In defining priorities, the following should be considered:

-- State of the science base and the feasibility of each proposal, in terms of the state of knowledge and available resources;

-- Demand for the information likely to be generated by the study--the probability that it will be put to use--in terms of the potential impact on health care, the burden of illness, and various ethical issues.

-- Likelihood that the research will test assumptions on which current policies and delivery practices are based, provide the basis for developing new options for health services delivery, or provide the means for monitoring performance of the health care system;

-- Provision of flexibility for innovative proposals;

-- Participation in the process by those responsible for providing the research support, as well as those directly associated with the problems and needs;

B12. Planning and coordination of applied health research activities among DHEW agencies need to be strengthened, although the Department's present pluralistic approach to the support of clinical applications and health services research must be maintained.

B13. Planning and coordination can best be accomplished by establishment of a committee at the level of the Office of the Secretary, which includes representatives of all DHEW agencies involved in applied health research. The committee would serve generally to monitor the Department's commitment to provide an applied research capability, evaluate priorities and identify gaps and duplications of effort. Authority for program-specific research should remain within each agency. General health services, which is primarily the function of the National Center for Health Services Research, should be maintained as such, and the Center should become the operational arm of the committee. Coordination must also be increased between HEW and private industry, particularly in the area of developmental research."

Commentary

These principles highlight a perspective to which we give strong support and endorsement: "A strategy must be adopted for funding health research which includes provision for...expansions for the support of clinical applications, clinical trials and health services research activities without diversions of funds from fundamental research." (B7) We find it highly significant that this theme has been stated repeatedly in these principles, at the October 1978 conference, and in the panel reports of that conference as well--not only by fundamental science constituencies, but also by those concerned with applied health research. We express support both for the idea of guarding against erosion in fundamental research support, and for a strengthening of applied research--two related, but distinct concepts.

It is not easy to "strengthen" various components of applied research, however, and the principles do not discuss adequately how to do so. It is not simply a question of directing more money to specific research areas, although in some fields where the science base is strong, that may be adequate. More typically, there are problems in the institutional and personnel base of these fields that require resolution. In epidemiology, biostatistics, and toxicology, for example, there appear to be shortages of adequately trained scientists to meet existing needs. Although the reasons for these shortages vary, a simple increase in program funding for research in these areas without addressing the more fundamental institutional and personnel problems would be an inadequate strategy.

We also suggest that the applied health sciences require a long-term commitment to developing their fundamental concepts and tools. Principle B6 accurately pinpoints selected methods and measurements in need of refinement. Such "discipline building" requires durability of support over the long term, not only for research on specific problems and health needs, but also for meeting the personnel and institutional requirements of these disciplines.

Several of the principles (B3 especially) discuss the responsibilities of the health research agencies to the regulatory, care and policymaking agencies. Our Committee did not try to resolve what such responsibilities should be, or the parallel issue of whether these agencies should themselves develop stronger research capabilities. However, there was a consensus that the principles should not attempt to settle these questions prematurely in the absence of the detailed consideration which issues of this magnitude deserve. We suggest, for example, that the demand process outlined in B3--whereby the regulatory, care and policy agencies simply request certain research from the research agencies

and the latter are required to respond--is too simple and perhaps not feasible. There is no question that mechanisms need to be established to assure the development of the knowledge base required by these agencies to meet their responsibilities. But as the principles now stand, it appears that many nuances of this need have not been analyzed. For example, as regards the important research needs of the regulatory agencies in particular:

- it is possible that requests from regulatory agencies could overwhelm the research agencies and seriously compromise existing programs in such agencies;

- the knowledge requirements in the regulatory agencies are of many types--some very short term and immediate, others more fundamental (as in the search for appropriate animal models); it is likely that research mechanisms to address such different needs will have to be more multi-faceted than the principles suggest;

- the role of the private sector in providing more of the knowledge needed by, for example, EPA and FDA merits exploration.

A comparable list related to the health care and policy agencies could also be formulated. We have not discussed such issues in detail, but suggest that these questions and others require more thorough consideration--perhaps through the permanent planning mechanism suggested earlier--than is possible here before DHEW acts on these issues.

The principles call attention to the issue of validating preventive, diagnostic, and therapeutic measures before their introduction into the health care system. Much concern has recently been expressed about the problem of premature dissemination of developments; this Committee shares the concern. However, the principle addressed to this problem (B4), if taken literally, raises the possibility of the reverse problem. This all-but-explicit endorsement of routine recourse to randomized clinical trials not only challenges the conventional pattern of innovation in human medicine but will require considerable expense and, frequently, a great deal of time. In the end, much needless consumer expenditure for ineffective treatment may be eliminated, but a substantial front-end cost and considerable political controversy will be encountered. The probable intent of the drafters of this principle, and our view as well, is that studies on risks and benefits especially are required before the <u>widespread</u> introduction of a new procedure throughout the health care system. We are concerned about the spread of fads, and the development of habits in health care practice which lack a solid basis in scientific evidence. But the clinical studies

needed to produce such evidence must not be structured as obstacles to responsible clinical innovations; it is obviously in the public interest to encourage a steady flow of well-considered new developments. We suggest, therefore, that Principle B4 be tempered to address this perspective. The critical issue in our view lies more at the level of widespread utilization and reimbursement for new procedures than at the point of innovation.

In Principle B7, the notion is raised of using the health care dollar to help support large scale clinical trials, health services research, and other applications research. We presume this specifically means that the budget of the Health Care Financing Administration (HCFA) is a possible source of support for the applied health sciences, particularly if an increase in funding for this area is intended. The Committee finds this a reasonable suggestion--as a way to find new funding sources, to protect the fundamental sciences, and to link health care services and research more closely. An agency such as HCFA has a clear need for the information generated by applied inquiries about the services and systems it is supporting. The "health care dollar" goes beyond HCFA, however, private insurers and purchasers of health care (employers and employees) also have a potential role in supporting applied research and should be involved in discussions about this important financing possibility.

We are not certain about the preferred locus for large-scale clinical trials. These efforts benefit from a special blend of managerial expertise and scientific/technical competence, and require substantial funding--a combination that is not easily found. In undertaking such large-scale studies, it may be desirable to have joint ventures between the Public Health Service (PHS) and HCFA; for example, HCFA might well finance such studies and PHS conduct them. This is yet another policy option in need of study, and with some urgency. Agencies and institutions that pay for health services are likely to be increasingly concerned about what they are paying for in health care, particularly as worries about cost control grow.

Additional comments on specific principles follow:

- In cautioning that needed increases in support for "clinical applications, clinical trials and health services research should not erode support for fundamental research" (B7)--a principle which we endorse--it would be useful to recognize that the costs of each of these neglected areas vary widely. Large scale clinical trials may <u>each</u> cost many millions of dollars, (even though some of these costs are not associated with the

trial itself and are instead costs of care that would have been purchased anyway), whereas needed support of health services research, for example, is probably of a smaller magnitude than the aggregate cost of all desirable clinical trials.

- The value placed on peer review and excellence (B8) is endorsed by this Committee here and elsewhere.

- Principles B9 and B10 speak inadequately to problems of knowledge dissemination and technology transfer. It will be important to consider not only the dissemination of information, but also methods to assure proper and adequate utilization of new information in the field. This area, too, will require much effort over a long period of time.

- As regards "consensus development approaches," (B10) we assume these refer to recent NIH workshops on health interventions of uncertain or controversial risks and benefits (such as mammography). Although we agree that it is useful to bring together experts and experienced practitioners from time to time to summarize the current state of knowledge regarding specific health technologies, such meetings are no substitute for needed evaluation of health care interventions through rigorous research.

- The task of establishing priorities for applied research is discussed in Principle B11. We agree that many of the factors listed are proper issues to be weighed in setting priorities. The principle, however, is silent on the subject of who is to set the priorities. Without further discussion of this fundamental question, the principle avoids a very important set of issues that pertain, among other things, to this current planning activity. As we ask in Part II of this Report, after all the principles are in hand, what set of decision-makers will actually set the five-year research priorities for DHEW?

- Coordination of health research in DHEW is the subject of B12 and B13. Although the phrase "pluralistic approach" is ambiguous, we are generally supportive of the idea that research areas may be overlapping. Multiple approaches to similar problems are often productive. We are more concerned about the recommendation for a

Secretary-level committee to "coordinate" DHEW applied health research and the confusing suggestion about NCHSR becoming the "operational arm" of this Committee. We do not have sufficient insight into the administrative workings of DHEW to know whether or not this is a good idea. Perhaps the Committee would be better located in the Office of the Assistant Secretary for Health or elsewhere. In any event, this issue requires more careful study--preferably within the context of our suggestion for a permanent health sciences planning mechanism--and should not be settled prematurely in a statement of principles.

- The very last sentence of the last principle in this section states that "coordination must also be increased between DHEW and private industry, particularly in the area of developmental research." In our view, one of the major deficiencies of the principles is that public-private sector relationships are neglected. This single statement is inadequate for the importance of the issue. For example, the principles do not speak to the role of private industry in supporting large-scale clinical trials, in developing prototypes of medical products or devices based on advanced research carried out in academic or government research institutions, or in promoting health education and information dissemination. A fully adequate five-year plan for health research should include consideration of such matters.

C. Principles Regarding Health Regulation and Promotion

"This focus addresses the further development of the knowledge base necessary for regulatory decisions and preventive and control measures, the need to communicate effectively the nature and inherent constraints of the regulatory process, and the need to explore more structured and creative interactions between the health research agencies and the agencies responsible for health promotion and regulatory activities.

C1. Principle--The knowledge base necessary to protect the public health through the establishment of preventive measures, the conduct of health education and promotion activities, and the formulation of regulations must be further developed.

C2. Progress in health regulation and promotion requires an increased commitment to health research, both basic and applied. The behavioral sciences are an integral part of this effort.

C3. Support of fundamental research should continue to be the responsibility of NIH. The NIEHS and NIOSH have a special role in adding to and exploiting basic knowledge to improve our understanding of environmental and occupational hazards. The support of applied research that is necessary to convert limited information into scientifically acceptable regulatory and control options should continue to be pluralistic.

C4. New approaches must be explored to assure that the knowledge needs of the regulatory agencies are clearly and systematically communicated to the health research agencies and that health research is responsive to these needs.

C5. The public should be informed about the nature of the regulatory process, its strengths, and limitations, including the constraints imposed upon it by the frequent need to make immediate decisions based on available knowledge.

C6. Research (most of it basic) should be supported to determine the most effective means of health promotion and disease prevention, the best ways of transmitting information to effect changes in behavior, and how specific measures can be most effectively directed to different populations. Programmatic approaches for health promotion research should stress the total range of interrelated health conditions.

C7. The public must be encouraged to improve health by adopting preventive measures and, when necessary, altering nutrition, environments, lifestyles, and behavior to reduce the risk of disease, disability, and premature death. Such measures are more beneficial and less costly in the long-run than pursuing curative or rehabilitative approaches. More reliable data bases should be developed to help guide such decisions.

C8. Research is needed to provide broader and more accurate data bases and assessments of well-being and effective social function as well as more accurate measures of the burden of illness.

C9. In order to promote better health and to prevent illness, intensive and innovative cooperative efforts must be pursued by the government, health professionals, voluntary health organizations, and others in the private sector so that the public may be better informed and educated about the application of knowledge gained through research.

C10. An expanded effort to support large-scale epidemiologic studies should be initiated as an approach to health protection. Such studies should include the natural history of disorders and the identification and quantification of both risk and protective factors.

C11. The Secretary, DHEW, should be empowered to maintain an undesignated research fund to be allocated by an interagency coordinating committee with representation from NIH and the regulatory and service agencies for the purpose of dealing with urgent problems that arise unexpectedly in the health protection and health promotion areas."

Commentary

Regulation and promotion of health are activities not ordinarily considered when one reviews the research enterprise. However, as concern grows about the toxic effects of environmental pollutants, and the importance of health-enhancing behavior receives greater recognition, it is clear that further research in health promotion and regulatory activites is necessary. We endorse the explicit attention in the principles to these issues.

The historical basis of regulation in health has been well described. The sanitary movement of the 19th century occupied the energies of several generations of reformers and social activitists and may have been largely responsible for the declining death-rates at the end of that century. Child-labor, sewage disposal, food and drug safety, work-place hygiene and water purification are only some of the health issues that required regulatory intervention. The conflict between the health of the consumer or worker and the desire for profit by the manufacturer has often been reconcilable only by regulation. Health promotion has an even more ancient basis, and in many societies, behavior detrimental to health has been recognized and actively discouraged--alcohol and drug abuse, over-eating, and smoking being prime examples of harmful behaviors. Given this historical perspective, we find it most appropriate that these DHEW principles give explicit attention to the interplay of health with regulation and behavior.

Several themes related to the principles which we wish to highlight with approval include:

- The appreciation that we need to know more about personal behavior related to health, and how to modify such behavior. We have learned well that admonitions to behave in certain health-enhancing ways are not enough. A great deal more research, much of it basic, will be required if modifications in personal lifestyle are to be encouraged successfully.

- The discussions of epidemiology, biostatistics, and other population-based sciences as principal tools in the area of health promotion and disease prevention.

- The specific attention to the research needs of the regulatory agencies.

- The recognition that improved measures and methods are needed within the science base of health promotion and disease prevention. For example, survey data of many types are needed, and we require more sophisticated measures of health status than existing mortality and morbidity indices provide.

The discussion of the research needs of regulatory agencies is particularly important. There are unfortunate past examples

of a lack of cooperation between research agencies and regulatory agencies. There needs to be a resolution of whether or not regulatory agencies should increase their research capacity at many levels in order to meet their responsibilities. As noted earlier, we urge that DHEW examine this issue very thoughtfully before a decision is made, and that such examination distinguish clearly between fundamental and applied research. We have already stated our view that fundamental biomedical research should remain the responsibility of NIH and, to a much lesser extent, ADAMHA. However, we reiterate that there is need for further analysis of the responsibility of various DHEW agencies, including the regulatory agencies, for fundamental research in fields other than the biomedical sciences.

The last sentence of C3 states: "The support of applied research that is necessary to convert limited information into scientifically acceptable regulatory and control options should continue to be pluralistic." We are not sure what "pluralistic" means in this context, but we are sympathetic to the idea that regulatory agencies must be able to meet their needs for the results of short-term, applied research, on the condition that funds for such work are specifically provided and earmarked.

Principle C7 discusses the goal of modifying personal behavior to enhance health. We find this perspective important and support the need for basic research about behavior (C6). We contend, however, that social institutions also figure prominently in health-related behavior. For example, the food and advertising industries share the responsibility for the poor nutritional and diet patterns in the population. In efforts to prevent disease and promote health, we should encourage institutional change also, and not focus exclusively on individual behavior. Principle C9 speaks of the need for "intensive and innovative cooperative efforts...by the government, health professionals, voluntary health organizations, and others in the private sector" to educate and inform the public about new knowledge regarding health promotion and illness prevention. These "cooperative efforts" should also be directed towards changes in organizations and social institutions so as to create a milieu favorable to personal health.

Additional comments on individual principles follow:

- C5 correctly suggests that the public needs to know more about the process of, and rationale for, regulation. We note that in the current climate of debate and controversy regarding regulation, there are those who argue for less regulation and attempt to trivialize the adverse effects of regulated products; there are others who argue that regulatory agencies are not adequately protecting the public interest. In this atmosphere of tension resulting from such divergent views, the public needs to be better informed about the concepts and

practices of regulation, and the data on which regulatory decisions are based, so that disagreements about various aspects of regulatory activity may be debated and resolved wisely.

- The first sentence of C3 does not acknowledge the presence of ADAMHA in supporting fundamental biomedical research, or the need for additional consideration regarding the administrative locus for fundamental research in disciplines other than the biomedical sciences (see page 29 for a fuller discussion of this issue).

- C6 mentions "interrelated health conditions." This phrase acknowledges that, particularly with the emergence of chronic disease as a major part of the burden of illness, health problems are now understood to have multiple causes, and multiple connections with other health and even social problems. Consequently, a wide range of approaches is required for their diminution or prevention. However, multidisciplinary and interdisciplinary studies often do not fit conveniently within the disease specific institutes of NIH, for example, and may not be funded adequately due to such institutional barriers. The mechanisms for funding multidisciplinary research within DHEW need additional attention.

- C10 is, as noted, most valuable for its strong endorsement of epidemiological studies. However, the term "large-scale" may be misleading. Some epidemiological research is quite small in scale, but also in need of greater support. We note that epidemiological teaching and research in medical shcools are particularly neglected.

- Principle C11 proposes that an "undesignated research fund" be maintained by the Secretary for urgent needs related to health protection and health promotion. We suggest that the problem of establishing mechanisms to fund research that responds rapidly to suddenly appearing needs without abandoning quality standards is a more complex one than can be solved by a single committee or mechanism. Flexibility and more rapid response to new needs for research would be valuable in all areas—not solely health protection and health promotion. Deciding on the best mechanism to provide such flexibility will require a detailed review of, for example, the grant and contract award processes in all health-related agencies, alternative mechanisms for allocating flexible funds (using what priority system?), and the strengths and limitations of centralizing control of such funds in the Secretary's office; other loci might be more appropriate.

In our view, several areas were not given adequate prominence in this set of principles:

- The importance of establishing specific funding mechanisms for epidemiological studies.
- Research on the processes and practices of regulation. What are the benefits, costs, and conflicts inherent in regulation directed towards protecting the health of the public?
- The need for better "early warning systems" to identify occupational and environmental health hazards.
- The tension between private economic interests and the protection of the health of the public in a democratic society.

D. Principles Regarding Research Capability

" This focus addresses the need to assure that the resources, both physical and human, necessary for the conduct of research are available.

D1. Principle--Present research capabilities must be sustained and enhanced to assure future health gains.

D2. Programs designed to enable capable young women to compete successfully for research support should be provided.

D3. It is essential to bring into the research community new investigators with new and innovative ideas and to provide them with support to capitalize on opportunities. Individual peer-reviewed project grants, institutional fellowships and some new and innovative highly targeted programs are some of the mechanisms through which this could be accomplished. This is necessary to train and maintain the next generation of investigators. Research careers must be kept competitive with other pursuits in order to attract and train outstanding individuals.

D4. Talented individuals should be encouraged to pursue research interests in the early stages of their scientific and medical careers. Support at both the pre- and postdoctoral levels are necessary to accomplish this.

D5. Recruitment of young researchers to areas of greatest national need and scientific promise must be encouraged. Mechanisms that may aid in doing this are individual research career development awards, individual and institutional fellowships, and new and young investigator and academic awards.

D6. Clinical research depends upon providing opportunities for promising clinically trained individuals with aptitude for research to develop into independent investigators as well as for Ph.D.'s to enter clinical and other research disciplines.

D7. It is essential to provide opportunities to individuals who have just obtained health professional or research degrees to embark on academic and/or Federal research careers.

D8. Investigators of proven productivity should be able to depend on stable research support during their demonstrably active careers and should be buffered against radical short-term changes in policy.

D9. Programs to encourage the research training and research grant support of minority investigators and institutions should be continued and expanded.

D10. Programs designed to enable capable young women to compete successfully for research support should be provided.

D11. Programs to support multidisciplinary, collaborative research approaches must be available.

D12. Programs need to be established and/or expanded in research areas currently undermanned, such as biostatistics, epidemiology, demography, sociology, environmental and occupational health.

D13. Major research activity at research and academic institutions such as health professional schools, graduate schools and Federal and National laboratories, such as the NIH intramural research program, must be fostered, and research support provided in a stable manner. Performance of research is inseparable from the learning process, enriching both directly and indirectly the quality of health care:

D14. Investigators and institutions need some measure of stability and should be buffered against radical short-term changes in policy.

D15. It is essential to provide an adequate level of long-term support to assure stability of training programs concerned with fundamental and clinical research in biomedical and behavioral disciplines.

D16. Physical resources must be renewed and maintained. This requires giving attention to both equipment and facilities, since both are major determinants in the productivity of capable scientists.

D17. Resources should be conserved through sharing, methods developed to stimulate greater ingenuity in the design and construction of laboratories and buildings, and support for health science libraries and other biomedical communication resources continued and expanded.

D18. Grants for improvement of existing and construction of new animal facilities must be continued and expanded, as well as support for breeding and base-line characterization of needed research animals. The potential for preservation of stocks of organisms in the dormant state should be explored, as should reliable methods for frozen maintenance centers and distribution of stocks when called for.

D19. A continuous assessment of ongoing needs and resources should be pursued. Additional studies of the future of junior faculty positions are needed, and the employment model used in estimating needs for the number of trained individuals and disciplines should be expanded into a more complete job market. Data to evaluate and monitor the condition of existing facilities and equipment are needed."

Commentary

In general, we endorse this set of principles, although as a beginning, it would be wise to distinguish clearly among human resources, physical resources, and animal resources (see paragraph D19) because different considerations come into play in planning for each. Human resources are the most important and merit the highest priority in funding.

A number of problems make it difficult to plan for meeting human resource needs. The course of science is difficult to predict, and the talents of well-trained scientists can be flexibly and diversely employed. The idea that one can predict "needs" for a particular number of persons trained in a specific scientific discipline is, accordingly, inappropriately narrow. Yet we can forecast confidently that a steady flow of young scientists is required to maintain the vitality of the nation's research enterprise. Current data on demand for and supply of scientific research personnel are inadequate, and we have insufficient and incomplete knowledge about the course of research careers in health science. The needs for research personnel seem intimately linked to the level of federal funding; yet short-term fluctuations in that level must not be matched by attempts to narrow and widen the flow of trainees, for research training is a long-term enterprise. The time interval from the decision to enter a research career to the achievement of independent investigator status is about a decade. The full effect of moves to encourage or discourage entry into research careers will not be felt for 15 to 20 years, but the effect will be irrevocable. If we make the mistake of skipping a generation in the 1980s, health research will suffer severely, for young scientists are "not only essential for the future, they are also indispensable for the work that has to be done today," as the Report of the President's Biomedical Research Panel asserts.

Scientific research is a career that requires particular talent, temperament and motivation, as well as specialized preparation. Many early aspirants to research careers soon find that they do not possess one of these attributes and willingly turn to other pursuits. It is thus important both to expose potential research scientists early to the opportunities as well as to the rigors of the career in order to help them make an appropriate choice, and to facilitate their progress along a pathway that may be long and difficult.

The principles are valuable in calling attention to the special needs of training clinical investigators (D6) - an issue that has been raised by the observations of the NAS-NRC Committee on National Needs for Biomedical and Behavioral Research Personnel and by the Institute of Medicine's Committee on Health Sciences

Policy. There is agreement, so far, only on the existence of a problem--namely, the apparent undersupply of candidates qualified to pursue clinical research careers. There is less agreement on the reasons for this situation, which may include:

- the disincentives of relatively low stipends for trainees and the burden of payback requirements;
- the economic unattractiveness of a career in research compared to the practice of specialty medicine;
- the current shortage or instability of research grant funding--especially for new (hence unproven) investigators;
- the dearth of research oriented internships and residencies for newly graduated MD's and especially for M.D.-Ph.D. graduates;
- current public attention and policy emphasis upon needs for primary care;
- a significant motivation toward non-research directions arising from the requirements of medical specialty board certification, and recent changes in medical school curricula.

Any or all of these explanations may have some merit. If the field of clinical investigation is to be strengthened, as we have adovacted, it will be important that the relative significance of these various factors be analyzed, and that policies to enhance the attractiveness of this field be implemented.

In the interim, there are some readily apparent countermeasures that are worth pursuing. There appears to be a consensus that the Medical Scientist Training Program of NIH is effective in attracting talented medical students into clinical research. Some successful attempts have also been made to provide clinical experience in medicine to Ph.D. scientists with the aim of focusing their attention on clinical research. Such programs deserve further analysis to determine their benefits and limitations.

We note that--just as we are calling attention to the field of clinical investigation--the principles themselves (especially D12) attempt to specify certain scientific areas requiring nurture. Based on the aforementioned NAS Committee on National Needs, we would agree that toxicology, biostatistics, and epidemiology appear to be shortage areas. Conversely, there seems to be an oversupply of doctoral level biomedical

scientists in relation to available positions for such individuals, due primarily to demographic factors that are leading to no growth in higher education enrollments, and to budgetary decisions that have slowed or stopped growth in research funding. However, it is important to recognize that, for all these areas, there are many difficulties in determining national needs in scientific fields, or in developing evidence for oversupplied or undersupplied areas. The match between available positions and personnel is a complex mixture of economic, social, and educational factors. For example, oversupply may mean both that the nation is not providing adequate career opportunities in needed areas for properly trained scientists, and/or that there is a true excess of trained individuals given both the needs and opportunities in a given area. As regards the reverse problem, we suggest that if an undersupplied area is identified, remedies will likely include a combination of training support and project support--often in combination--to ease the shortage.

Several of the principles speak about institutional stability, which the Committee views as a central issue in research planning. We believe that a national strategy for health sciences research should explicitly and clearly recognize the role of the institution in which individual investigators work and its needs for stability. It is not enough to ask for stability of funding for investigators. It is also essential to provide for institutional integrity and freedom from disruption by radical short-term changes in policy. The university assumes responsibility for the support of teacher-investigators when they join its faculty, and assumes responsibility for seeing that facilties and equipment are provided, responding in good faith to governmental requests for research. The institution deserves some buffering from the inordinate number of program redirections, eliminations, new initiatives, and expansions that have characterized recent federal programming. Institutions also require reimbursement for the indirect costs of research (overhead) which truly compensate them for the actual costs of supporting the research and the research environment e.g. animal facilities and libraries. Training programs (D15) share this need for stability. Program and funding disruptions lead to inefficiencies in the use of funds, to depleting institutional resources, and ultimately to discouraging the talented individuals who would undertake research careers.

The principles might usefully recognize the significant part now played by the Biomedical Research Support Grant (BRSG) in fostering institutional stability and helping the research institution to fulfill its obligations. Such funding provides

start-up support for new investigators and exploratory work on untested research strategies, smooths out some of the discontinuities that seem inevitable in the current pattern of project grants, and economizes on total system effort in the assessment of small-scale innovative inquiry.

A possible contradiction threads throughout the principles. The call for stability of support for productive investigators during their active careers, and the notion of providing for new investigators and fresh talent may be in conflict, particularly if an environment of sharply limited research support exists. This conflict is not easily resolved and is therefore identified in Part IV of this Report as an area requiring careful study.

One issue not adequately discussed by the principles is whether training programs--like aspects of applied research--should be mission-oriented or problem-specific. Our view is that training, even more than research, should not be "targeted" on current health problems although it is clearly appropriate to select certain disciplines for special support. The long interval required for training scientists suggests that efforts to focus training programs on special health problems is usually inappropriate, except, perhaps, in a very few scientific areas or in the later stages of training.

The need to provide adequate physical facilities and equipment is appropriately highlighted in the principles (D16 and D19). However, we require better information on facilities and equipment to plan rationally for their renewal (as emphasized in D19).

Although not specifically mentioned in the principles, we suggest that it is important to maintain clinical research centers. The metabolic ward model is not the only or even the most significant type of needed clinical research facility; nevertheless it serves an important purpose, particularly in studies of chronic disease that require environmental and dietary control and continuous monitoring of patient functioning. Other types of clinical research facilities are important for other types of problems--e.g. the scatterbed facilities for the study of acute, severe disease, and outpatient facilities for ambulatory conditions.

We are also concerned specifically that the animal resources required for selected aspects of health sciences research be maintained and increased as needed. The October Conference Panel report on research capability outlined this issue adequately, pointing out the need for the ready availability of genetically controlled, well-characterized stocks of a wide variety of research organisms, the need to support improved animal research facilities

and to search continuously for new animal homologs of human diseases, and the importance of maintaining existing animal models—e.g. non-human primates.

We comment additionally on three specific principles:

- We strongly endorse the prominence given in Principle D11 to multidisciplinary, collaborative research programs, particularly in applied research. Health problems, by their very nature, are often complex and are not readily resolved by unidisciplinary research. There is need to facilitate the multidisciplinary collaboration advocated in the principle, so that scientists may join in generating research programs that are of a scale suited to the dimensions of many of our health problems. This principle, incidentally, probably belongs in another section, perhaps "unifying concepts."

- Principle D17 states that: "Resources should be conserved through sharing, methods developed to stimulate greater ingenuity in the design and construction of laboratories and buildings, and support for health sciences libraries and other biomedical communication resources should be continued and expanded." The need for greater sharing within and among institutions of expensive or precious resources is a highly significant issue. Computer centers and animal research facilities are examples of such resources that should be shared to avoid unnecessary expense and duplication of effort. Much could be learned from the field of high energy physics in which expensive facilities are routinely and efficiently shared. More specifically, as this principle is phrased, it misses the point of the panel comment from the October conference, namely: ingenuity in design <u>in order to achieve greater flexibility in usage</u>. Also, it is unclear why only libraries and communication resources are singled out specifically for "continued and expanded support," but not for the application of ingenuity or conservation through sharing.

- D19 calls for the expansion of the employment model used to estimate research personnel. Although this sounds plausible, the expansion may be difficult and costly because of the dearth of data; and the result may not be worth the effort since the fragmentary evidence available suggests that all but a small fraction of biomedical and behavioral research related to health is performed in universities, federal and state governmental agencies. D19 also asks for additional information on the number of junior faculty positions needed in the future; we agree that such data are desirable.

E. Principles Regarding Unifying Concepts

"This focus deals with concepts that provide the basis for consideration of the philosophic base for public funding of health research and the interactions among the different parts of the research continuum necessary to move from the development of new knowledge to the application of research findings. Considerations include the need: to reaffirm the Federal support of health research; to fulfill public expectations regarding a return on the public's investment in health research; to communicate effectively the nature of the scientific process, its limitations and benefits; to develop bridges between fundamental and applied research; and to explore new interactions and processes.

E1. The Federal Government should support health research, and the rationale for such support should be clearly presented to the public which makes such support possible.

E2. There should be long-term stability in the funding of health research, but within this stability of funding there should be sufficient flexibility to allow for a rapid response to promising new research opportunities.

E3. Fundamental research should be defined broadly and the need for a science base (or basic research) should be reaffirmed.

E4. The funding and training of young investigators are essential to the future of health research.

E5. The decision-making process in the formulation of health research policy should be both open and accountable.

E6. Health research policies should be formulated in the light of a gradually evolving set of ethical and legal standards for the conduct of such research.

E7. Efforts to involve previously underrepresented groups in health research should be continued.

E8. DHEW agencies can perform an important matchmaking function by identifying mutually relevant and reinforcing research efforts.

E9. The criteria for setting health research priorities among various fields and approaches should not be rigidly defined, but should consider a variety of factors ranging from research opportunity to social need."

Commentary

These principles, which touch on many basic philosophical and policy issues, represent a substantial condensation of earlier versions of the principles, particularly as compared to the September 15, 1978 draft. The process of wringing out possible controversy resulted in language that is so general that it is difficult to be sure which choices are being advocated. Thus, the comments in this section are intentionally less directed to the explicit language of the draft principles than is true for the four preceding sections.

These current unifying principles are altogether commendable. What is left out is the means by which health research can be made more effective; especially by bridges between fundamental and applied research.

The draft gives no hint of how poorly informed we are about the processes of creative discovery and its fruitful applications. There is little room to quarrel about the overall relationship of research and innovation, nor about the high return of benefits from health research investment. When it comes to finer details like the resilence of research motivation in the face of external regulation (accountability) or the optimal structure and allocation of research effort (by discipline or by immediacy of application), we are relying on common sense and personal belief. We should not confuse intuitive pronouncements on how best to do science with the better validated products of scientific inquiry.

Appropriate policies for scientific research could perhaps be elucidated through further systematic analysis of the history of research. Except for the work of Comroe-Dripps, most of our current knowledge about the scientific process is fragmentary anecdote. In many cases we would not know how to answer either "could the time to discovery be shortened?" or "could fruitful applications have been accelerated?"

A key fact that must illuminate public expectations of contemporary health research is the difference in the research tasks posed by infectious versus the degenerative diseases which are our principal challenge in health today. The latter entails laborious search for deep knowledge of the human organism far more complex and less accessible than the biology of the known parasites. Nevertheless, the stock of fundamental biological knowledge acquired during the last 25 years has implications for revolutionary advances in the improvement of health during the next 25.

Even for applied research, a cardinal principle should be: "How can we best exploit the most creative minds--how to identify, encourage, guide, and support rather than frustrate them? And how can we improve the most effective interplay of knowledge gained through both fundamental and applied research?"

The articulation of fundamental science with applied science requires the most thoughtful attention. Linkages are usually accomplished through a variety of institutions, notably the research universities and medical research institutes. In the name of accountability, many obstacles are placed in the way of such organizations and of efforts at integration; and little is done to relieve innumerable fiscal, political, and administrative stresses that tend to splinter into microscopic projects the activities that should be integrated.

At some point, accountability as an end in itself can defeat the underlying purpose of the enterprise. The greatest pressures for accountability arise when grants are based on overly narrow precepts that do violence to the service institutions that receive and administer the grants. Accountability should be tempered to meet reasonable public aims and to avoid fraud, not to lockstep each grantee to the bureaucratic rigor of the funding agency.

The linear continuum of research expressed throughout the principles is an over-simplified mode. In many cases, clinical observations or puzzles arising from practice have inspired the most fundamental inquiries. For example, the work that led to the discovery that DNA was the hereditary substance arose out of the most practically motivated efforts to develop vaccines for the prevention of pneumonia. The progressive compartmentalization of scientific inquiry into narrowly defined projects tends to frustrate the feedback along the research continuum which is necessary for the most vital exploitation of unpredictable opportunities.

The facilitation of communication among investigators who work at different points on the research continuum is preferable to centralized mandates for the pursuit of specific objectives by different actors. Funding mechanisms should facilitate productive interactions and communications between basic and applied scientists and between investigators in different disciplines, and should enhance the interplay of research and training.

Beyond these more general observations, we wish to discuss several individual principles:

- E1 discusses both the role of the federal government as the major supporter of health research and the need for taxpayers to be provided with a cogent "rationale" for such support. We wish to amplify this important principle. There are few alternative sources of support for health research outside of the public sector, as private sector research pertinent to health is governed by considerations other than national need. And although the private, non-profit sector provides important support for selected areas of health research, its resources are in no way comparable to public sector capabilities. The importance

of this federal role and responsibility cannot be emphasized enough. We also suggest that the rationale for such support has in fact been presented on numerous occasions--most recently in selected appendices to the President's Panel Report. We nonetheless endorse strongly the idea that the rationale should be presented frequently, in order to provide the public with an informed basis for deciding whether its trust and support are merited. This does not suggest that the public is wavering in its support of health research. Indeed, it is essential to this entire planning activity to recognize that public support for research in general, and research to improve health in particular, is high and strong. This is one important reason why policymakers should have little reluctance to take actions to extend and enhance the health sciences.

- We support the philosophical allegiance to openness in policy formulation, as stated in E5. Both the courts and public opinion support this approach. Yet we have much to learn about mechanisms to provide for such openness in a constructive fashion. The limited success of the October 1978 Conference on Health Research Principles demonstrated that much careful attention is needed to the problem of how best to structure public involvement.

- E6 quite properly asserts that research, like all other social endeavors, should comply with existing legal and ethical norms. If this principle speaks indirectly to regulation of research, we refer back to and endorse the discussion of regulation at the end of the discussion on the Fundamental Research principles.

- Principle E9 states that "the criteria for setting health research priorities among various fields and approaches should not be rigidly defined, but should consider a variety of factors ranging from research opportunity to social need." An earlier version of this principle which appeared in the September 1978 draft was more illuminating, and we therefore restate it below:

  "Investments in areas of health research should be guided by many factors, including research opportunity, burden of illness measures, demographic trends, public perceptions of the relative importance of different health areas, current state-of-the-art, previous investment and return experiences, near term potential for new breakthroughs, problem areas identified through

disease surveillance and investigation of disease outbreaks, interrelationships with other research problems, benefits which may accrue by elimination or improved treatment of disease, the perception of those concerned or involved in dealing with social problems or needs, and a continuing retrospective assessment of health benefits attained through research expenditures. The relative importance of the above factors in allocating resources should vary between basic and applied research."

PART IV

TOPICS FOR FURTHER STUDY

The Committee wishes to call attention to an additional concern that transcends earlier comments about the process being used to develop the plan and about the individual principles. Several significant aspects of the federal role in health research have not yet been raised in this activity. There are also some issues that have been raised, but have been inadequately discussed or resolved.

Given the time available for this review, the Committee cannot analyze these issues or make specific suggestions about how they should be handled in a comprehensive five-year stragety for health research. Nonetheless, it is our view that these issues are central to the task of planning for health research. To exclude careful consideration of them in the current activity runs at least two risks: 1) that federal support of health research for the future will be planned without having addressed these fundamental issues--a shortsighted approach that overlooks challenges and problems facing the research enterprise; and 2) that these issues will be resolved or settled all too quickly in the last stages of this planning activity, with only superficial analysis, and therefore without an adequate basis for effective and wise action. As we have noted earlier and frequently, the Fall 1979 deadline for the completion of the plan does not seem likely to allow for the detailed inquiry these issues require--a possiblity that adds to concerns expressed earlier about the process being followed to construct the plan.

Several of these fundamental issues are listed below:

- <u>The development of an appropriate equilibrium between the requirements of stability for support of research, and the need to provide opportunities for young investigators</u>. What are the approaches that may provide for continuity of support for proven senior investigators and yet not so limit the opportunities for newly trained scientists that the flow of bright young investigators slackens? Is it possible, through a variety of support mechanisms and through careful administrative tuning, to achieve both goals?

- The administrative locus for, and financial support of, clinical trials, particularly large-scale trials. Which institutions are best suited to organize and conduct large-scale clinical trials in a way that provides for both scientific and management expertise? Is is possible for several DHEW agencies to participate in the support of clinical trials? How are such trials to be financed, and is there a role for combined private and public support of such activities? How can new sources of funding be developed? By what guidelines are specific trials to be selected from the universe of all possible trials?

- An institutional home for modern "public health research." Is there a need for a new administrative locus for a constellation of scientific areas which are currently scattered and undersupported throughout DHEW--including epidemiology and biostatistics, health services research, and prevention research? Given our growing understanding of the need for, and contribution of, research in these areas, how best can these areas be nurtured and invigorated?

- Organizational aspects of the NIH, including both its intramural and extramural program. An incisive review of DHEW's health research activities should include analysis of such items as: the desirability of categorical institutes; the effects of the categorical labels on public understanding of health research and on support for non-categorical missions such as those embodied by the National Institute of General Medical Sciences; the relationship of the NIH intramural program to its extramural activities; the limited peer review of the intramural program; and the differences in career paths afforded scientists in the intramural as compared to the extramural program. An analysis of such issues, however, should occur within a framework that recognizes the value of the NIH and defends its administrative and functional integrity. The flexibility of the NIH must be maintained and its successful record acknowledged and reaffirmed.

- The coordination of public and private health research activities. What should be the relationship of these two sectors, particularly regarding development needs growing out of fundamental and applied research? What is required to take a scientific advance and translate it into an effective application in health care, particularly

when the public sector is reluctant to become involved in development activities, and private industry tends to work only on those products likely to be profitable? How can the federal government better stimulate innovation, the transfer of innovations into practice, and the ongoing evaluation and monitoring of new interventions?

- The introduction of new diagnostic, therapeutic, and preventive procedures into the health care system. At what stage are clinical trials to be mounted and under the auspices of which agency? In practical terms, how can monitoring and evaluation of technology occur in a way that does not stifle innovation but also assures that widely used procedures are of known risks and benefits? What is the role of third party insurance reimbursement mechanisms in assuring that only procedures or products that have received appropriate clinical evaluation are reimbursed?

- Providing for an adequate pool of clinical investigators. The Committee notes with concern the growing difficulty in attracting and retaining an adequate pool of researchers who span the fields of fundamental research and clinical practice--a critical link essential to the mutual stimulation of both areas of science. What are the causes of the diminution of physician interest in the field of clinical investigation? How might training programs, career paths, support mechanisms and other factors be modified to increase the number of highly competent clinical investigators? What are the institutional barriers to vigorous exchange between and among the fundamental and clinical sciences and how might these be eased?

- Systematic sharing with other nations of ideas, information, and resources pertinent to health research. Are current mechanisms adequate for such international cooperation and coordination? If not, how may they be improved?

We also reiterate that several issues raised in the principles should be studied further before action is taken. These include such matters as the relationship of DHEW research agencies to the regulatory, health policy, and health care service agencies; agencies; mechanisms within DHEW to coordinate applied health research; and the administrative locus for fundamental research in areas other than the biomedical sciences.

Research Needs for Research Planning

In coming years, planning for research will probably increase. Good planning for health research, however, is hampered by the lack of adequate information on a variety of issues which should all be weighed carefully in developing a plan. Some of these needs for "research on research" are listed below and are suggested as topics on which information should be collected in order to improve the data base for future planning:

- Past patterns of research support (including financial, institutional, and personnel dimensions) that have proved to be especially productive, as a guide to preferred patterns of support for the future. The Comroe-Dripps study is an excellent beginning for this area of study related to the biomedical sciences; for other fields, such as biostatistics, epidemiolgoy, and the behavioral sciences, such historical data need to be developed.

- Analytic work on the general problem of establishing research priorities, including the development of methods for assessing the state of a science and outlining areas of scientific opportunity.

- The burden of illness nationally and internationally, utilizing many measures beyond mortality to assist in establishing research priorities.

- The incentives and disincentives facing individuals who have elected careers in health research, particularly those in the area of clinical investigation.