Association of American Medical Colleges

Preserving America's Preeminence In Medical Research

Principles for the Support of Biomedical Research

Suite 200, One Dupont Circle, N.W., Washington, D.C. 20036

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Principles for the Support of Biomedical Research

Approved by the AAMC Executive Council September 1983

Principles for the Support of Biomedical Research

- The greatest scientific productivity occurs when highly creative investigators are provided with appropriate resources and work in an environment free of excessive demands from external regulation and directives.
- The identification of the most promising research areas and the determination of their important dimensions are in large measure a scientific judgment requiring highly knowledgeable experts in related fields.
- The need for additional knowledge requires a major emphasis on basic research.
- Free communication among investigators is the lifeblood of science; adequate resources and means must be available to facilitate that communication.
- The continuing replenishment of the pool of intellectual talent and the maintenance of the infrastructure of research institutions are essential.
- Funding for biomedical research by the federal government is essential and must be the principal source for the scale of effort currently established.
- Funding of the research enterprise should be predicated on long-term perspectives and should minimize sudden or wide fluctuations.
- Evidence of scientific merit in proposed projects should be the key determinant in decisions relating to the award of funds for research support.
- The terms and conditions of fiscal support should be compatible with and not seriously distort the administrative processes of recipient institutions.
- Investigators and organizations engaged in research must continually demonstrate an active sense of public and scientific accountability.
- Public expectations as to the benefits of investment in scientific activity should be realistic in terms of the unpredictabilities of research, and the substantial time lag between fundamental discoveries and their widespread impact on health problems.

The Problem

The evolution of the National Institutes of Health (NIH) into the world's most productive and prestigious biomedical research enterprise has been one of the important and remarkable developments in this country during the post-World War II period. Recent events suggest the favorable conditions that contributed to that phenomenon are changing. Most prominent among the forces influencing that change has been a significant modification in approaches to legislation under which the NIH has been funded and managed. Spurred in large part by dissatisfaction with funding levels for NIH programs in their areas of interest, both lay and professional leaders of many disease-oriented organizations have turned increasingly over the last decade to a responsive Congress. They have adopted a strategy of proposing new legislation as a means of satisfying their aspirations for greater visibility and support. This approach is epitomized by bills currently before the Congress that contain numerous specific directives to NIH which, if passed, would attain the relative permanence of statute. Conversely, the components of the NIH itself are moved toward relative impermanence because of the need for the periodic renewal of expiring legislative authorities, such as those for the Cancer and Heart Institutes. Given the almost infinite number of potential disease-oriented causes and the predictable competition among them for greater recognition, this circumstance creates a continuing opportunity for the expansion of set-asides, institutes, boards, task forces and programs. Over time, such legislation would create the antithesis of the broad, elegant authority for biomedical research, unencumbered by detailed directives, as enacted in 1944. The consequence would be an inevitable erosion and ultimately the destruction of the delicate balance between the political and scientific forces that has been and remains so crucial to the success of NIH.

The Elements of a Successful Biomedical Research Program

With the record of repeated accomplishments and the strong promise of continued productivity, it is essential that the environment in which the research enterprise functions continues to assure that the solid record of the past will be emulated, if not exceeded, in the future. Because scientific inquiry in itself is a dynamic process, the management of the program and the instruments provided for its management must also be dynamic in character. The elimination of some diseases as major threats, the emergence of new forms of illness and the ever increasing and changing knowledge base all must be recognized as developments to which the content and direction of the program must be adapted. At the same time, certain considerations, regardless of the time or the state of change, will remain essential to the well-being of both the nation's biomedical research enterprise and its primary instrument, the National Institutes of Health. Accordingly, the following characteristics deserve recognition by those responsible for or interested in the continued vitality of the NIH and its programs. This community includes scientists themselves, as well as administrators, legislators and leaders in the commercial and public sectors.

- The greatest scientific productivity occurs when highly creative investigators are provided with appropriate resources and work in an environment free of excessive demands from external regulation and directives.
- The identification of the most promising research areas and the determination of their important dimensions are in large measure a scientific judgment requiring highly knowledgeable experts in related fields.
- The need for additional knowledge requires a major emphasis on basic research.
- Free communication among investigators is the lifeblood of science; adequate resources and means must be available to facilitate that communication.
- The continuing replenishment of the pool of intellectual talent and the maintenance of the infrastructure of research institutions are essential.
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The evolution of this set of working principles over a thirty-year period has given the nation a highly effective model for the pursuit of an important social objective. Modification should be undertaken only on firm justification and after thorough examination of the possible consequences, lest serious harm be done to the integrity of the enterprise.

Background and History

Widespread and persistent public interest in extending the human lifespan and in enhancing the state of physical and mental well-being has prompted the establishment and maintenance of a very substantial medical research enterprise in this country. This phenomenon was predicated on the premise that only with new knowledge derived from a vigorous, diverse and high quality research effort could progress be made in reducing the toll of suffering and economic loss from cancer, heart disease, arthritis, and a host of other maladies. It was further premised that only the federal government could acquire and sustain the sizable financial resources necessary for such adventure.

These conditions and their exploitation in a responsible, visionary and cooperative manner by a small number of individuals from government, academe and the public resulted in the NIH. Its success can be attributed in large part to five unusual circumstances, all of which were essential. They were:

- the establishment and maintenance of a crucial balance between the political and scientific forces that influence the medical research program,
- the relationship between the NIH and research-intensive academic institutions,
- the provision and reaffirmation by the Congress of general authorities to the Public Health Service for the conduct and support of medical research,

- the insistence that scientific merit should be the preliminary determinant of funding, and
- the continuing appropriation of funds by the Congress for the NIH operation.

The first two involved the forging of significant and enduring relationships. One was the matching of the political appeal of categorical diseases with identified scientific opportunities. This relationship is epitomized by the nature of the names given most of the major NIH organization components (e.g., National Cancer Institute) and by the widespread use of expert scientific advisors for planning and evaluating research programs and for selecting research projects for funding. The establishment and persistence of this *modus vivendi* is as contributory as any other single condition because of its unquestioned influence on the congressional appropriation process. Not inconsequentially, it has been probably the greatest determinant of the productivity and quality of the agency. Unfortunately, it is also probably the most fragile. Either of two far less desirable possibilities could have occurred. The scientific community could have insisted on organizing not only the research but its funding and administration along the traditional lines of scientific disciplines (e.g., a National Institute for Biochemistry). Alternatively, those fighting the causes for specific diseases could have insisted that the enabling legislation require that the distribution of funds appropriated for research be made proportional to the comparative levels of disease-specific mortality or morbidity. That concept would tie the appropriations, at least in terms of visibility, much closer to their identified disease interests. The first approach obviously would have lessened the emotional impact generated by serious disease, meaning far lower appropriations for research. The second would have unquestionably wasted tax-derived dollars, because scientific opportunities and the incidence of disease frequently do not coincide. Instead a remarkably ingenious confluence of interests was evolved. It is most apparent in the two-tier advisory system that was established at NIH, in part by legislative mandate and in part by administrative action. The National Advisory Councils generally are composed of individuals having some identified association with a particular categorical disease and drawn from either the professions or the public, while the technical review panels, made up of individuals with established scientific reputations, are charged with the responsibility for assessing proposals for scientific merit.

The other essential relationship was a partnership between the NIH and the bio-scientific and academic communities, represented especially by the universities. While the purposes and activities of the partners are not identical, they have been highly compatible and a relationship has developed that has been generally characterized by a high degree of mutual

dependence and trust. Through federal policy and funding, this arrangement has permitted the public interest to be served by the best source for the generation of new knowledge required for the fight against disease while at the same time indirectly but definitely strengthening many institutions of higher learning. The public interest has been thereby enhanced in two notable ways.

The third contributing factor of great significance was the provision in 1944 of enabling legislation in Section 301 of the Public Health Service Act that was almost unique in its combination of no temporal or dollar ceilings and few directives to the NIH. It was ideally suited to contend with the unpredictabilities of pace, direction, opportunity and outcome in a research activity. These characteristics, so inherent in scientific inquiry, require unusual flexibility in the management of a research effort. At the same time, it was eminently clear that the NIH was to be a *health* agency, using science to fulfill its mission. The enactment and preservation for almost three decades of this elegant legislation reflected a remarkable degree of foresight and self-restraint by the Congress. The legislature disregarded methods previously adopted for dealing with more applied activities such as defense or commerce and selected one for the biomedical research program that recognized both congressional responsibilities and limitations. It enabled:

- the Congress to discharge its responsibilities through the processes of oversight and appropriations,
- the NIH to develop a flexible management concept and operation, based on high quality science, that facilitated and strengthened the nation's biomedical research enterprise,
- the scientist to pursue promising avenues of research, and
- the public to express its aspirations through the appropriation process and participation in the Advisory Council apparatus.

The fourth factor was the insistence by the agency, its advisors and the Congress that scientific merit should be the primary determinant in the allocation of research monies. This principle assured that the research supported had the highest probability of developing valuable new knowledge and offered the greatest likelihood for the most rapid and most effective improvements in the treatment of specific diseases. Fortunately, the insistence on merit as a keystone has been broadly based and unrelenting, because well-intentioned but scientifically deficient proposals for the solution of disease problems are often vigorously promoted.

The fifth major influence was the provision by the Congress of continuing financial support for the effort. Funds have been provided annually with strong bipartisan support after an extensive hearing process and with only general instructions as to their deployment.

Biomedical Research Accomplishments

As a result of these conditions, a vast increase has occurred in our understanding of the fundamentals of health and disease and the practice of medicine has been revolutionalized. Some afflictions, especially among the infectious diseases, have all but disappeared as major threats to our citizens and the knowledge base is well on its way to a level of development that will permit major assaults on more complex, chronic ailments. Scientific fields such as endocrinology, genetics, immunology, the neurosciences and virology abound with important discoveries that offer hope of earlier diagnosis or more effective measures for prevention or treatment of numerous diseases. At a time when the costs of health care are coming under increasing scrutiny, research leading to the prevention or cure of illness represents the most rewarding approach to control or reduce those costs.

In addition to the benefits which have accrued in terms of health *per se*, there have been two other highly desirable consequences. The first is the very significant return on the biomedical research investment that has occurred in non-medical areas. Such biomedical discoveries as freeze-drying and its application to food preservation, genetic manipulation and its uses in agriculture, laboratory instrument computers and contributions to the development of mini-computers, fiber optics and their growing use in telecommunications, and enzyme biochemistry in the development of new types of detergents have contributed significantly to the development of whole new industries. Second, the emergence of the large and high quality biomedical research endeavor in this country established the United States as the world's leader in this field. At a time when the Nation has lost some of its preeminence in other fields, our citizens can still point with pride to the maintenance of leadership in biomedical research.

Preserving the Enterprise

Despite all the accomplishments and accolades and the appearance of an undertaking of great permanence, the continued vitality of the NIH endeavor requires constant vigilance and protection. Preserving academic values under the aegis of public funding on the one hand, or adopting the special standards of public conduct in the very private research enterprise on the other, has not been easy in the United States or in any other free society. Yet the stakes for the public good are so high that every effort should be made to devise and institutionalize workable arrangements. Our current cultural emphasis on the short-term gain and the frequent failure to

distinguish between science and technology contain a constant threat to the well-being of the NIH. The sheer size of the effort with its heavy dependency on federal funding represents another point of great vulnerability. Even its own friends and benefactors when dissatisfied with their share of the resources or degree of visibility in its operations may inadvertently cause serious problems. It behooves all to whom these considerations are addressed not only to provide support for the continued integrity and vitality of NIH but also to exert restraint during periods of temporary frustration or dissatisfaction with day-to-day decisions or outcomes. Thus:

- Biomedical scientists should keep constantly in mind their responsibilities to the public that provides the funding and determines the character of the national environment in which the scientific effort occurs. Part of this responsibility is participation in education of the public about biomedical science, its capabilities and limitations.
- The public should recognize the limitations as well as the capabilities of scientific inquiry to assure a public climate of tolerance for the uncertainties of scientific effort.
- Individuals and organizations with disease-specific interests should consider possible negative impacts of their proposals for legislative mandates in specific categorical areas on the integrity and vitality of the NIH as a whole.
- Legislators' personal agendas should have as a high priority the preservation of that fine and difficult line between their representative and advocacy responsibilities and their fiduciary responsibilities as trustees of the nation's research enterprise.
- Administrators should recognize that facilitating the scientific effort while assuring adequate scientific, financial and ethical accountability will always remain their paramount task.
- The NIH should extend and formalize their current procedures to receive, evaluate and appropriately publicize proposals by advocacy groups for modifications in program content, emphasis or priority.

Only if these considerations are recognized and accepted will the rewards of the investment for better health be fully realized.