ON BIOMEDICAL RESEARCH POLICY IN THE FUTURE

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January 1989

P-7520

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Mr. Walden, members of the Science Policy Task Force, I am honored to be invited to appear on this panel and to offer my thoughts on future biomedical research policy. My perspective is that of an outsider with a longstanding interest in federal biomedical policy. I shall comment on ways in which I believe that developments in the U.S. health care system and other aspects of health policy are likely to impinge on biomedical research and the academic medical centers where much of it is conducted.

Factors Affecting Biomedical Research

I foresee five major related factors as having important effects on biomedical research over the next decade:

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[1] This paper includes the text of the prepared testimony of Albert P. Williams, Director, Health Sciences Program, and Director, RAND/UCLA Center for Health Policy Study, The RAND Corporation before the Science Policy Task Force, Committee on Science and Technology, United States House of Representatives, April 24, 1986.

It also includes his responses to five questions submitted to him several months after his testimony.

- o The pressure to reduce and control federal deficits;
- o Persistent problems of controlling health care costs;
- o Changes in health care payment systems;
- o Growing alliances between academic medicine and the private, for-profit health care-related industry; and
- o Generally increased budgetary stringency in academic medical centers.

The pressure to control federal deficits seems certain to continue well into the 1990s, which means tight biomedical research budgets. Many proposals with scientific merit are likely to remain unfunded, the competition for existing funds will increase, and the search for new funding sources will become more intense.

Although substantial progress has been made in the control of health care costs over the past several years, the problem is going to persist, and it may adversely affect biomedical research funding, independent of the general pressure it places on the federal budget. Advances in medical science often involve expensive new technologies and some believe that one way to control costs is for the government to spend less on biomedical research.

New payment systems, such as the Medicare Prospective Payment System (PPS), capitated payment plans, and preferred provider organizations, provide disincentives for the use of expensive new diagnostic and treatment modalities, unless they are cost-saving. Of

course, there are some safeguards in these systems against inappropriate rationing of new technology, but it is difficult to design features that will discourage only wasteful use without also discouraging some beneficial use. In the current controversy over Medicare's payment of hospital capital costs, one of the important considerations is the potential effects on the development and diffusion of new technology.

One of the most striking changes in academic medicine over the past decade is in the attitudes toward relations with industry. In the early 1970s, investigators who accepted money from the pharmaceutical industry to fund their research were generally regarded as having "sold out." Today almost all the major academic research institutions already have or are exploring some form of agreement with the for-profit sector. This seems a very constructive change, but it will profoundly affect the incentives facing faculty researchers and, perhaps, the willingness of traditional funders of biomedical research to continue their support.

Although the new alliances with industry promise to alleviate some financial problems of academic medicine, there are many reasons to expect that these budgets will become progressively tighter: the growing concern over an excess supply of physicians seems likely to make public and private funding of medical education less generous. The federal government has already taken steps to cut back Medicare's payments for the so-called indirect costs of medical education, and there are proposals to reduce reimbursement for direct educational costs. Both demographic and economic factors are likely to reduce the

demand for medical education and thereby make it difficult for medical schools to raise revenue by increasing tuition. Increased competition among all hospitals for patients will hold down the prices that teaching hospitals can charge. Many teaching hospitals provide a disproportionate amount of care to Medicaid and charity patients; because funding for such care is becoming scarcer, teaching hospital budgets will be strained further. Finally, academic medicine will be forced to deal with the growing need to do more training in outpatient settings where there are no well-established means of covering costs.

Candidate for Policy Concern

These five factors seem likely to interact with one another to affect the biomedical research community in potentially adverse ways. I mention them, not to play the role of a prophet of doom, but rather to suggest problems on which policy should focus in order to maintain the healthiest possible research enterprise, given resource and other constraints. Likely candidates for policy concern are:

- Supply of research funding,
- Demand for research products,
- Requirements for research support,
- Allocation of research funds, and
- Directions of research activity.

Understanding their nature and origins seems essential to developing effective policy solutions.

Supply of Research Funding. The pressures from federal deficits and difficulties in controlling health care costs, combined with suspicions that research advances inevitably increase costs, will strengthen the hands of those who believe we spend too much on biomedical research. These arguments remind me of the Luddites, a group of workers in the early 19th century who tried to smash machines in order to prevent what they regarded as adverse consequences of mechanization. These latter-day Luddites, like their predecessors, are not entirely wrong in identifying some adverse effects of technological advance, but they too fail to balance costs against benefits and to seek ways to reduce the former and increase the latter.

Demand for Research Products. Technological advances have been stimulated by the same forces that have led to spiraling health care costs--reimbursement systems that almost unquestioningly paid for anything that the doctor ordered, no matter how uncertain or small the benefits. The strong incentives for using expensive medical technologies created a demand for new drugs, devices, and procedures. Efforts to control costs cannot be effective without reducing that demand, and consequently, the demand for the basic and applied research that produces them. By no means are all advances in medical technology cost-increasing, and many cost-increasing advances produce benefits that are more than worth it. However, it is hard to determine at the early stages of development, much less at the basic research stage, how a new

technological advance will affect health care costs. This seems almost sure to make the development of new medical technology riskier business and the commercial payoffs of biomedical research more uncertain. Ironically, this change will be occurring at a time when academe and industry are developing effective ways to work together.

Requirements for Research Support. As funding becomes tighter for academic medicine and for the agencies that have supported it, the level of argument intensifies over cost burden-sharing. Academic medical centers produce much of their education, research, and patient care in what economists call a joint production process, such that it is impossible to separate all the pure costs of producing each product. As a consequence, there are "joint costs" that must be allocated in some rather arbitrary way to one of the products; there is no one "right way" to allocate these joint costs, only more or less acceptable ways to those who are asked to pay. When all budgets are tight, everyone presses hard to pay less of the joint costs. The current debates over ceilings on indirect cost reimbursement and over Medicare payment of medical education costs in teaching hospitals are manifestations of this. I do not wish to enter what I regard as being a largely unproductive debate over whether biomedical research has paid more or less than its fair share of these costs in the past. However, it seems unlikely to me that more of these costs can be shifted to the patient care or education dollar in the future. Thus, I see little prospect for getting more bang for the federal biomedical research buck than in the past.

Allocation of Research Funds. The NIH peer review system has repeatedly withstood the attacks of its critics. However, I believe that it will face ever more difficult tests in the future, because of the inherent difficulties of allocating scarce resources to an ever-larger number of scientifically meritorious proposals. In a period of more generous funding, the consequences of small "mistakes" in peer review were less. The "pay line" did not exclude so many highly rated proposals, and if a solid proposal were not funded on first submission, it would often be funded on the next. Almost surely that is no longer the case; there are simply too many solid proposals. As budgets get tighter, some believe that peer review will become more conservative in its judgments, choosing more established science in lieu of more innovative research. I think we don't know how the system will respond. I do believe that it is certain that its tasks will become more difficult at the same time that they become more important.

Directions of Research Activity. The new alliances between academe and industry offer many new opportunities for stimulating research, but it is clear that industry's criteria for supporting research will be different from those to which the biomedical research community has been accustomed in the past. Some biomedical discoveries can readily be commercialized because they involve patentable products or processes. Other at least equally important discoveries are in the public domain from the time they are reported, because they are in the nature of new

understanding of physiologic or disease processes. The new knowledge becomes a public good rather than a proprietary product. Industry benefits from both classes of discovery, but companies should have to answer to their stockholders if they do not show a preference for investing in research that is more likely to produce commercializable, proprietary goods. Industry, for example, would be less likely to have supported the basic research that earned Drs. Brown and Goldstein the Nobel Prize in medicine than research that promised to lead to new patentable drugs. My point is not that commercializable research is any less important to society but that the new opportunities for investigators to share more of the financial returns of such research will increase incentives to do more of it and less of research that produces knowledge for the public domain.

Policy Implications

The problems outlined pose difficult challenges for federal biomedical research policy and health policy, more generally. I make no pretense of having solutions, but I think it is possible to identify some areas in which policy initiatives will be required.

The multiple pressures on research and health care budgets will make it even more important to have sound bases for determining which technologies are cost-effective and in which circumstances. Despite the very impressive advances in medicine in recent decades, there is strikingly little hard evidence on the efficacy of much of the health

care provided. And, the federal government invests very little in technology assessment, despite its large investment in basic knowledge generation and the large burden of health care costs it bears. Unless we produce better information about the effectiveness of new technology and eliminate wasteful use, I fear that the latter-day Luddites will carry the day, reducing the use of technology and cutting the budgets for the basic research that generates new knowledge.

The success of health care cost control efforts will depend in part on their ability to reduce the wasteful use of expensive medical technology. Waste reduction is unexceptionable, but it is extraordinarily difficult to design payment systems that will not also discourage investments in technologies that have benefits that outweigh their costs, that may even be cost-saving for the system as a whole but not necessarily for the provider or the one who pays for the bill. I expect there will be a continuing need to modify payment systems to ensure that they do not adversely affect important new technological developments.

I expect there is no better way to allocate biomedical research funds than on the basis of peer review, but that does not mean that the present peer review system is perfect. Indeed, even if it had worked perfectly in the past, it would work less well in the future because other things are changing. It will be important to analyze the process in order to improve it and to adapt it to future challenges. As funding becomes tighter, the consequences of peer review become more important.

The review process is regarded by some as very expensive, but it may be necessary to spend more on it in order to achieve more efficient allocation of biomedical research resources.

The biomedical research community has long viewed with great suspicion any suggestion that the federal government should try to influence the direction of its research. Instead it has admonished government simply to fund the best science, as judged by peer review. As long as NIH was the major funder of basic biomedical research, there was a strong case for such a *laissez faire* policy, but this situation seems likely to change as private industry plays a large role in funding academic research. NIH may need to be more attentive to balancing incentives for investigators. In particular, the financial incentives for institutions and individual investigators seem likely to attract more researchers to potentially commercializable pursuits, and NIH may need to focus more attention on ensuring that research producing knowledge in the public domain is not short changed.

Follow-up Questions
for
Dr. Albert P. Williams

1. With respect to considering future direction for government policy in support of the biomedical sciences, what should be the respective roles of the scientist, the public, and the government in the decision process for setting short- and long-term priorities in biomedical research? In public health?

The scientist, the public, and the government should have few fundamental differences in objectives with respect to the setting of priorities for biomedical or public health research. That is, each is presumably concerned with having research move in directions most useful for society. The problems in setting priorities and the differences between these three groups tend to arise because of the differences in their perspectives regarding the planning and predictability of scientific advance, and to the extent that advance can be predicted and planned, the practical consequences of having non-scientists involved in setting priorities.

Unfortunately, each group mistrusts the sophistication and the motivations of the other. The scientist generally fears that public or government involvement in setting priorities will cause the government's (and private foundations') resources to be wasted on research of little scientific merit, simply because it falsely promises advances in areas of high social concern. The public fears that scientists are too strongly motivated by parochial concerns and the vogues of their community and are relatively unconcerned about the practical applications of their research, particularly in areas of high social concern. The components of the government are split according to their constituencies: Agencies that deal closely with the scientific

community and regard themselves as part of that community (such as the National Institutes of Health) feel that research resources are best allocated on the basis of judgments by scientific peers. Other agencies, such as the Office of Management and Budget, tend to be concerned about the incentives that drive the scientific community and want a stronger role for themselves and the non-scientist in setting priorities.

I think that public policy toward the support of biomedical and public health research would be improved by better communication and more collaboration among the scientific community, the public, and the government. The public should try to articulate its priorities more specifically. Scientists should try to respond as specifically as possible regarding the avenues of research that offer the greatest promise to serve those priorities and should be very candid about the limitations of current knowledge. Government should focus on evaluating existing programs and developing new ones to promote better science and to improve its contributions to the solution of high-priority problems.

2. It has been indicated that it is often difficult to predict the next major breakthrough in research. From your perspective, is it a desirable, long-term goal that the U.S. maintain leadership in all fields of biomedical research or should we build our strongest areas?

In responding to this question, I think it is important to distinguish between research whose results are in the international public domain, virtually from the point of discovery, and research that yields proprietary benefits. For the former, which includes most of the research that the federal government seeks to fund, it should make very little difference where discoveries are made, and we should logically be as pleased with a breakthrough by foreign scientists as we are with one

by U.S. scientists, except for concerns of national pride. For the latter class of research, we need to be concerned about effects on the U.S. international competitive position. It may make a lot of difference to the health of certain U.S. industries, such as pharmaceutical and medical technology firms, if other countries take the lead from us in certain areas. However, the government has generally not been directly involved in funding this class of research, and we have relied on the profit motive to stimulate private initiative in this area.

We should certainly be concerned about maintaining an environment conducive to our keeping as fast a pace of advance at the frontiers of science as any country in the world. We currently enjoy a lead position in many, if not most, scientific areas, but the magnitude of that lead has decreased and will continue to decrease as other countries develop their scientific communities. Because of the nature of breakthroughs, we can expect to lose a few "races," to continue with the metaphor suggested by this question, but we should not allow ourselves to drop in the "overall standings." If we start dropping back, it should be a signal to us that something is wrong, perhaps in our science education, perhaps in the incentives we offer scientists, perhaps in some other area.

3. In your view, to what extent should criteria beyond science, such as specific national health needs and other societal goals, play a role in decision-making about biomedical research support?

As indicated by my answer to the first question, I think health needs and other social needs should influence the way we spend public resources on biomedical science. This does not mean that we should fund

mediocre science, just because it has some relevance to these needs. Nor does it mean that "applied" science should receive a higher priority than "basic" science. It does mean that both the needs and the quality of the science should receive weight in the decision process.

4. In addition to the factors judged to be important for maintaining progress in biomedical research, what factors are important to assuring that we also reap the economic benefits of the ensuing technology? How can the government, academia, and industry work together more effectively to accomplish this goal?

At this point, too little attention and too few resources are devoted to the assessment of the real, as opposed to the theoretical, value of new medical technologies. In the absence of rigorous analysis to assess the value of new technologies and in the presence of health care financing systems that insulate most patients from the financial implications of much technology use, we almost surely engage in uneconomic uses of medical technology. That is, when the value of doing something is uncertain but someone else is going to pay the bill, chances are the technology will be used. I think we need to mobilize public resources to assess the value of new technologies to avoid wasting collective resources--either public or private--on uneconomic and ineffective applications. Information about the effectiveness of new technologies is truly a "public good" on which we have spent too little.

I am not sure about the most effective and efficient way to finance the analysis needed to produce this information. Obviously, the producer of a proprietary technology reaps benefits from a positive finding of value, but many new technologies, such as new surgical procedures, are not proprietary in nature. Those who pay the bills for medical care would presumably reap savings from better information on the effectiveness of medical technologies because they would not pay for

ineffective applications. Thus, while it is difficult to determine exactly how the financial burden of technology assessment should be spread across the various classes of beneficiaries--technology firms, government, third party insurers, etc.--it seems clear that the federal government would be a large beneficiary and a logical candidate for bearing a substantial share of the burden.

5. From your perspective, how will present and future biomedical research and technologies impact on the cost of medical care? Who should bear the cost? What will be the overall consequence?

How the new technologies impact on cost will depend on how well we are able to assess their value and eliminate ineffective and inefficient use. I expect and hope that research will yield new information and new technologies whose benefits to health will far outweigh what they add to the nation's cost of health care. Some new technologies should also result in cost savings. I believe that those who currently pay for health care would be willing to pay the bill for such new technologies, if their value can be proven. In the absence of such proof based on sound assessments, I expect there will be increasing reluctance to pay for new technologies or for the research that produces them.