The Role of Biomedical Research in Health Care Reform

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The national debate about health care reform presumes a simple trade-off among quality, cost, and access. Most Americans, who are already insured, instinctively fear that expanding access, although desirable, will inevitably lead to higher costs or reduced quality for everyone. This quandary is inevitable only if the focus is on cutting the costs of delivering today's health care, rather than on improving the quality and bringing down its costs for tomorrow. The critical issue that reformers are ignoring is innovation. In spite of the powerful potential of innovation to reduce health care costs, skewed incentives in the current system have caused some innovations to be used in cost-increasing ways, leading many observers to believe mistakenly that medical innovation necessarily increases costs. But this is a dangerous mistake in logic. Successful reform must encourage innovation and create strong incentives for advances that will drive down costs while enhancing quality.

Productive innovation in health care can and should be pursued on many dimensions. Improved outcome studies and measures could enhance the understanding of what treatments work and for whom. Advances in information systems could improve the ease with which physicians stay abreast of state-of-the-art treatments and outcomes in specific circumstances. New types of facilities could improve the quality and efficiency of health care delivery. Most importantly, biomedical advances could lead to an ongoing stream of new treatments and cures for widespread or chronic diseases.

The past savings from biomedical innovations in terms of both dollars and quality of life have been significant. For example, in the 1930s, tuberculosis was a common disease, affecting more than one American in a thousand (*1*). There was no effective treatment, so patients were sent to sanitoria for months or years. Without the biomedical innovation that prevents the disease, Americans would now face the bill for maintaining a quarter-million patients in long-term care, without improving their health. The annual cost of treating polio if a vaccine had not been found is estimated at $30 billion per year. Health care reform should recognize the notable economic gains of biomedical innovation in reducing the cost of health care, just as other industries recognize the role of research and development for cost reduction and quality enhancement. Biomedical innovation, however, is significantly undervalued in the current debate about health care reform.

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Why Biomedical Innovation Is Undervalued

There are four errors in logic that lead reformers to undervalue biomedical innovation. First, biomedical advances have been so rapidly incorporated into standard practice that we have become desensitized to them. Second, the accounting of benefits from biomedical innovation is poor. Third, the emergence or discovery of a new disease makes improving health a moving target. And fourth, the practical, clinical payoffs from basic biomedical research are unpredictable. The benefits of biomedical innovation must be appropriately valued in the health care debate. By failing to do, we do ourselves and our children an economic and medical disservice.

Rapid advance in biomedical science. The benefits of biomedical research have come so rapidly over recent decades that the human and economic returns are often underappreciated. Lithium treatment for manic depression has saved over $145 billion in hospitalization costs since its introduction less than quarter century ago (*2*). Potassium citrate treatment for preventing kidney stone recurrence saves an estimated $400 million to $870 million per year (*2*). A new vaccine to prevent hemophilus influenza type b (hib) disease, the leading cause of bacterial meningitis in the United States, saves an estimated $350 million to $450 million annually as well as preventing tragic neurological damage and death among infants and children (*2*).

Despite such examples, critics argue that biomedical innovation raises costs. However, because the pattern of scientific advance is uneven, efforts to halt cost-increasing innovation could have the opposite effect. The coevolution of costs and benefits in many medical treatments reveals the importance of research innovation. Consider, for example, the treatment of bleeding peptic ulcers. Initially, most people who developed such ulcers died. Surgical techniques were developed to remove the affected area; these surgeries reduced mortality, but were expensive. An understanding of the role of stomach acids prompted the development of antacids that reduced the incidence of peptic ulcers. But surgery for acute cases was still necessary until the development of newer drugs like Tagamet and Zantac, which, over 15 years, reduced the rate of surgeries by 59% (*3*). Many patients, however, suffered recurrences after drug treatment. Further biomedical research showed that ulcers can be complicated by a bacterium, *helicobacter pylori*, a discovery that invited a simple cure with inexpensive antibiotics and saves $600 million to $800 million annually in treatment costs (*2*, *4*). This progression is typical of biomedical innovation: An incurable and expensive disease is initially treated with invasive surgery, then by progressively effective drug therapy, and finally by an inexpensive cure or vaccine. It would have been a mistake to freeze the process of discovery at an expensive intermediate stage by not investing in the next discovery.

Poor accounting of benefits. Reformers should consider both costs and benefits, not just short-term costs. The cost of health care should be measured by dollars spent and by illness endured; the benefit should be measured by the value of health, that is, prevented, cured, and mitigated disease, not by doctors' visits and hospitalizations, which are actually costs, not benefits. Unfortunately, benefits are often vaguely attributed to quality of life rather than to specific avoided costs of previous treatments, or to willingness to pay. For example, though recent new pharmacological treatments for cystic fibrosis and multiple sclerosis may be criticized as expensive, the considerable costs of those drugs are more than offset by a decrease in hospitalization and treatment costs. Pulmozine, a genetically engineered drug recently developed for cystic fibrosis, produced a net treatment cost reduction of 25% (*5*). Similarly, Betaseron, a new drug for multiple sclerosis, has been demonstrated to reduce hospitalization rates by 52% (*6*). Diseases that once plagued the population and are now prevented by vaccines—like polio, measles, and hepatitis—simply disappear from the ledger and are never accounted as health benefits or reduced health care costs. Tooth decay rates dropped 60% when fluoride was added to the water supply (*7*); $10 billion a year in dental

bills was saved in the 1980s from fluoridization and other advances (*8*), but the savings from investment in this research is not amortized to recognize its perpetual benefits.

Health as a moving target. Although the benefits of biomedical research can be great, they can also be difficult to measure because the definition of health changes over time. While biomedical innovation has eliminated diseases that once devastated the population such as smallpox, polio, and typhoid fever, new diseases emerge, like AIDS, Legionnaire's disease, Lyme disease, and toxic shock syndrome. Therefore, rather than ask why overall health care costs have not gone down as a result of biomedical innovation, it is more appropriate to ask how much more they would have gone up without such progress.

Unpredictable payoffs. In addition, it is hard to know where research will make its next contributions. Discoveries often unexpectedly apply to diseases far afield from a researcher's initial area of interest. Studies of DNA serendipitously led to drugs for cancer chemotherapy, herpes, and autoimmune diseases (*9*). The recombinant DNA revolution that is producing safe and new blood products has come from the study of bacteria (*10*). And the best hints so far about AIDS have come from the study of cancer (*11*).

Reformers may be uncomfortable with the unpredictable nature of biomedical innovation, and with a lack of linkage between a specific investment and specific need. But unexpected benefits are just as real as anticipated benefits and should be accounted for; they may outweigh not only the investment but the expected benefit as well.

Goals of Innovation

The real aim of biomedical research, achievable often only after long-term investment, is the prevention of disease and the maintenance of health—not the application of expensive Band-Aids. The goal should be not simply to extend life, but to promote good health and longevity, while reducing the cost of achieving them.

Critics characterize biomedical innovation only as an effort to cure rare diseases or invent heroic, expensive high-tech ways to extend the lives of critically ill people who have no cure and no hope. But as many of the previous examples demonstrate, that characterization is wrong.

Other skeptics argue that everyone must die of something eventually, and preventing or curing one disease simply provides an opportunity for a person to confront another disease. They point out that fast, inexpensive deaths at a young age are replaced by long, expensive deaths later in life. But cheap death does not necessarily increase social welfare. This criticism undervalues the economic benefits of health. For example, no one would dispute that there were gains to society by dramatically reducing the historically high risk of maternal death during childbirth. While such deaths were cheap and quick, it is clearly better for all concerned if surviving children have mothers to raise them. Their argument also ignores the fact that the cost of enabling a long, healthy life may be much less than the cost of caring for a chronically sick life. Some biomedical innovations directly reduce the cost of chronic or lingering death from a given disease. Once thousands of infants died each year of hyaline membrane disease, a condition now treated successfully with an inexpensive, easy-to-administer spray which saves over $70,000 in average hospital costs per baby (*12*). Rh antibody testing and intrauterine transfusion has resulted in a 96% survival rate of fetuses with maternal Rh incompatibility (*13*). Both of these are examples of low-cost treatment or prevention that enables normal healthy lives for infants who would previously have died in spite of elaborate attempts to save them.

Appropriate Incentives and Reform

The puzzle that confounds reformers is that in spite of this overall goal of simultaneous cost reduction and quality enhancement, innovation appears to drive up costs. But this appearance is misleading and is based on incomplete analysis. Where innovation has led to increased costs, it is largely due to the historical lack of incentives for cost control and almost complete lack of incentives for cost-reducing innovation (*14*). Skewed incentives were created by practices such as cost-plus reimbursement for facilities and equipment, pay-per-procedure reimbursement for physicians, the empowerment of insurers to profit by denying claims rather than by negotiating good value with providers, the lack of outcome and price data that would enable referring doctors and their patients to make informed choices when alternatives are available, and public policy that prevents the exit of inefficient providers. All of these skewed incentives have led to belatedly recognized problems such as the oversupply of expensive capital equipment (like magnetic resonance imaging machines and helicopters), excessive use of technologies that extend life without restoring health or quality of life, referral of patients for laboratory tests or procedures from which referring physicians profit, and performance of complex procedures (such as heart transplants) at hospitals in which the medical team has too few patients to become expert or efficient (*15*). Reform will be successful only if it corrects the incentives so that the power of innovation will work toward the goal of cost reduction.

With corrected incentives, profits from innovations will accrue only when innovation results in lower costs or improved outcomes. Indeed, the recent increase in cost consciousness and partial correction of past skewed incentives are already having dramatic effects on innovations in the biomedical development pipeline. Companies developing new treatments are pouring funds into products expected to provide dramatic cost reductions. While there will be a time lag before many of these new treatments are available, if strong incentives for innovation are understood, the benefits in improved health and in cost reduction will be substantial.

The Role of Public Investment in Basic Research

Stimulation of biomedical innovation requires both public investment in basic research and incentives for private investment. Unfortunately, there is often a lack of appreciation for the long-term benefits of biomedical research compared to other national needs. For example, last year when the Congressional Budget Office (CBO) estimated the consequences of a proposed reduction in the budget for the National Institutes of Health (NIH), it briefly noted that the decrease "could have adverse effects on biomedical research . . ." with no mention of the consequences on future health or health care costs. In contrast, the CBO offered a two-page analysis of the consequences of reducing by three the total number of aircraft carriers and airwings (*16*).

Despite the elusiveness of a comprehensive analysis, many examples of recent breakthroughs or potential future advances could have been cited. Recent biomedical innovation has relieved hemophiliacs from the terrible toll of AIDS by the development of synthetic clotting factors. Occurrence of measles went down from over 200 cases per 100,000 people to fewer than 1 per 100,000 after introduction of a vaccine (*17*). Newborn screening and treatment for hypothyroidism now prevents lifelong mental retardation for thousands and saves $200 million to $400 million per year (*2*). It is not clear how long it will take to control cancer, heart disease, AIDS, and Alzheimer's disease, but in the meantime, the costs are high: It is estimated that if the onset of Alzheimer's disease could be delayed just 1 year, $5 billion would be saved annually (*18*).

Though it may be difficult to establish the optimal level of research investment, there is evidence that the United States is underinvesting in basic research. This year

the NIH budget will not keep up with inflation. Of the grant applications selected as scientifically promising, fewer than one in four were funded last year (*19*). But, if basic research is so cost-effective, one may ask, why isn't it fully supported by the insurance or pharmaceutical or biotechnology industries? Because scientific inquiry can take years and lead in unexpected directions, the potential financial returns on any single scientific investigation may not justify the investment in the short run. Like the construction of interstate highways, biomedical innovation pays for itself times over in the aggregate, but it is too big an endeavor for any private investor. The provision of this kind of public good is an appropriate role of the government.

Although biomedical innovation was ignored in the Clinton health plan in Congress, there has been strong bipartisan support for increased investment in biomedical research as part of health care reform. Many members of Congress are recognizing the importance of training scientists and physicians, maintaining research hospitals and facilities, and continuing the flow of new discoveries. Despite the current dwindling of discretionary spending, several senators and representatives have proposed a Medical Trust Fund which would provide 1% of health care insurance premiums for biomedical research. The fund is based on the principle that just as industry invests in research with a profit motive, some part of health care reform should invest in biomedical research with a goal of cost saving and quality improvement.

After the inevitable political compromises to enact health care reform, it is critical that the resulting policy includes appropriate incentives for cost-reducing innovation and adequate public funding for NIH to support basic biomedical research. Federal support for biomedical research, which has focused on the benefits to health, should incorporate a realistic accounting of the contribution of innovative research to cost control as well. Past biomedical innovation has made major contributions in advancing medicine and significant contributions to cost reductions in spite of skewed incentives. With corrected incentives, the promise of future biomedical innovation to reduce costs is enormous. Only innovation will enable the dramatic and sustained cost reductions required for successful health care reform.

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The Paradox of Critical Mass for Women in Science

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A minority group (especially one that has traditionally been discriminated against) is easily marginalized when only a small presence in a larger population; its continued presence and survival is in constant jeopardy, requiring outside intervention and assistance to prevent extinction. As the group's presence and level of participation grows, at a particular point the perspective of members of the minority group and the character of relations between minority and majority changes qualitatively. In theory, the minority is increasingly able to organize itself and insure its survival from within and effects a transition to an accepted presence, without external assistance, in a self-sustaining process (*1*). The discrete point at which the presence of a sufficient number brings about qualitative improvement in conditions and accelerates the dynamics of change is known as "critical mass" and has been defined as a strong minority of at least 15% (*2*). Change, without struggle, however, is less likely than conflict with determined resistance. Under certain conditions, an organizational transformation culminates in minority group members achieving and retaining positions of real power and authority that were previously beyond their grasp (*3*).

To evaluate the dynamics and effects of these transitions for the problem of increasing participation of women in science, we studied 30 academic science departments in five disciplines (biology, chemistry, physics, computer science, and electrical engineering), comparing those departments that had been relatively successful in graduating female Ph.D.'s to those that had not (*4*). We also compared departments where a critical mass of women existed to departments where it was lacking.

A key finding was that as the number of women faculty members in a department increased, they divided into distinct subgroups that could be at odds with each other. Senior female scientists typically shared the values and work styles of older men; their narrow focus failed to meet the needs of most younger women. In contrast, some younger women (and a few men) struggled to create an alternative scientific role, balancing work and nonwork issues.

The scientific role thus bifurcates along generational and gender fault lines. These developments have significant unintended consequences for the socialization of female scientists, for example, the availability of relevant role models. As long as the relatively few women in academic science were willing to accept the strictures of a workplace organized on the assumption of a social and emotional support structure provided to the male scientist by an unpaid full-time housewife or done without, issues of women in science were not attended to. A modest increase in the numbers of women in science, without a change in the structure of the scientific workplace, creates a paradox of critical mass.

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