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Setting Biomedical Research Priorities in the 21st Century

David B. Resnik, JD, PhD

Setting biomedical research priorities is one of the most important issues in health policy and ethics because it has broad implications for the advancement of medical knowledge, the improvement of clinical practice, the promotion of public health, and access to health care. For example, funding research on the human immunodeficiency virus (HIV) can enhance our knowledge of HIV; improve the treatment, diagnosis, and prevention of HIV; and increase access to health care for HIV patients. But since neither the government nor the private sector has an unlimited supply of money to spend on research and development (R & D), determining how to slice the research funding pie raises social and ethical questions related to justice and fairness.

Most of the publicly funded biomedical research in the United States is sponsored by the National Institutes of Health (NIH), which had a $27 billion budget in 2002-2003. In the last 5 years, the NIH budget has nearly doubled.[1] Although the US government spends a great deal of money on biomedical research, private corporations spend more. In 2001, the companies belonging to the Pharmaceutical Research and Manufacturers of America (PhRMA) spent $30 billion on R & D, and companies belonging to the Biotechnology Industry Organization (BIO) spent $15.6 billion on R & D.[2-3] Seventy percent of the clinical trials conducted in the US are industry-sponsored.[4] Any realistic policy that addresses research priorities must come to terms with the fact that private industry outspends the public sector when it comes to biomedical R & D.

How Biomedical R & D Priorities Are Set in the United States

The economics of medical product development determines how pharmaceutical and biotechnology companies establish their funding priorities. According to industry estimates, it takes an average of $800 million and 10-15 years to develop a new drug, medical device, or biologic and bring it to the market.[5] Since a patent on a new product lasts 20 years, a company will have 5-10 years to recoup its R & D investment while the product is still under patent. Once the patent expires, the company will lose its exclusive control over the product and its ability to make a significant profit. Although pharmaceutical companies tend to have relatively high profit margins (ie, 10 percent or more), they also take significant economic risks when they develop new drugs. Only 33 percent of new drugs are profitable, and very few drugs become "blockbusters," like Viagra or Prozac. Companies also

frequently must withdraw profitable drugs from the market, due to adverse effects or litigation.[6]

Given these economic conditions, it is easy to see how private industry decides upon allocation of its biomedical R & D funds. Basically, pharmaceutical and biotechnology companies set R & D priorities based on market potential, liability costs, the scope of intellectual property protection, market lead time, the expected time from the laboratory to the market, and other factors that affect the profitability of a research investment. As a result, they tend to shy away from investing their funds on basic research, on rare diseases, on diseases with low consumer demand, or on drugs that will take a long time to get to the market or will have potentially high liability costs. Given these guidelines, private industry's R & D decisions can leave large gaps in our medical knowledge and may fail to promote the interests of all people in society. For example, 90 percent of the money spent on biomedical R & D focuses on conditions responsible for only 10 percent of the world's burden of disease.[7] Moreover, many of the drugs prescribed to children have not been tested on pediatric populations.[8]

Fortunately, the NIH fills in these gaps in medical knowledge and biomedical research. The NIH, established by the US government in 1887, consists of 27 different institutes and centers, such as the National Cancer Institute (NCI), the National Institute of Allergy and Infectious Diseases (NIAID), and the National Human Genome Research Institute (NHGRI). Its mission is "to acquire new knowledge to help prevent, detect, diagnose, and treat disease and disability, from the rarest genetic disorder to the common cold."[9] The NIH has more than 100 study sections, which review grant proposals and make recommendations to the NIH Advisory Council. In deciding how to prioritize research proposals, study section members consider several factors, including, (1) the proposal's impact on the burden of disease, (2) the proposal's potential contribution to biomedical science, (3) the qualifications of the researchers, and (4) institutional support for the proposal.[10]

To determine the burden of disease, one must balance and weigh a variety of factors, such as the incidence of the disease, the mortality rate of the disease, the degree of disability caused by the disease, the impact of the disease on life expectancy, the social and economic impacts of the disease, and public health considerations. Since value judgments enter into the weight and balance one gives these factors, the NIH solicits public input from elected officials, professional and scientific associations, disease advocacy groups, and special conferences, workshops, and review panels in assessing the burden of disease and establishing its research priorities. In addition, the NIH has established a Council of Public Representatives that provides the NIH director with advice on funding priorities.[10]

How Biomedical Research Priorities Should Be Set

Although private corporations tend to set their funding priorities based on profitability, one might argue that they should also consider their social

responsibilities when allocating their R & D funds. Private corporations have social responsibilities because they are accountable as moral agents in society and make decisions that have a tremendous impact on the economy, the environment, culture, and human health.[8] Pharmaceutical and biotechnology companies should exercise their social responsibilities by funding research to reduce the burden of diseases that affect people in developing nations and by sponsoring research on rare diseases, such as Huntington's disease or Tourette's syndrome.[11] They should also be willing to conduct research on pediatric populations, provided that they adhere to appropriate safeguards and regulations.[12] Pharmaceutical companies should, like the NIH, solicit public input and advice relating to their funding priorities. They should consult with many of the same groups that provide advice to the NIH, such as professional and scientific associations and disease advocacy groups.

While the NIH's system for setting biomedical research priorities is generally fair and effective, it also has some weaknesses. First, interest group politics can undermine both the fairness and the effectiveness of the system. Well-organized and well-funded disease advocacy groups can exert a disproportionately strong influence over funding priorities and can skew the research agenda. As a result, some diseases may not receive their fair share of research funding. Advocacy groups can also undermine the progress of biomedical research by urging the NIH to support research that lacks scientific merit, by deterring the NIH from committing funds to long-term projects or basic research, or by applying a political litmus test to research proposals. Second, prejudices, the "old boys network," and other biases can also adversely affect the fairness and effectiveness of priority setting.

In order to diminish these potential weaknesses, the NIH should seek the appropriate balance of public and expert input. It should give a fair hearing to proposals that lack the support of powerful interest groups; and it should establish procedures for overcoming the biases that can affect even well-designed systems. The NIH should maintain a strong commitment to funding basic research, research on rare diseases and conditions, and research on new and emerging diseases. It should listen carefully to public opinion but it should not allow its funding priorities to wave back and forth in the political winds.

Public-Private Cooperation

Major challenges in medicine and public health require public-private cooperation. For example, no single country, pharmaceutical company, or humanitarian organization can deal with the HIV/AIDS crisis in sub-Saharan Africa. Although this crisis continues to grow worse, the international community is beginning to see some meaningful cooperation among governments, multinational corporations, and humanitarian organizations. Developed nations, such as the US, have pledged to devote additional money for research, treatment, and prevention in Africa, and pharmaceutical companies have discounted their drug prices to make HIV medications more affordable. Governments must work with humanitarian organizations towards the goal of eradicating the spread of HIV. Governments can,

for example, fund basic research, while private companies can develop useful products and applications. Developing nations and humanitarian organizations can improve the health care infrastructure, while developed nations can contribute economic and medical resources.

The Medical Profession's Role

Physicians should take an active role in setting biomedical research priorities by advocating for fair and effective allocations of public and private biomedical R & D investments. Physicians should encourage pharmaceutical companies to make socially responsible funding decisions. Although it is often difficult to affect decisions made by large, multinational corporations, physicians can have considerable influence over pharmaceutical companies, especially when they focus and organize their lobbying power. Physicians should also help government agencies determine funding priorities and lobby the government. They should provide information and advice to the NIH and serve on study sections and advisory boards when asked.

The Council on Ethical and Judicial Affairs of the American Medical Association (AMA) has not issued any opinions dealing with biomedical research priority setting. However, the AMA's *Principles of Medical Ethics* lend support to the physician's role as an advocate for fair and effective research priorities to promote the advancement of medical knowledge, the betterment of public health, and increased access to care.[13]

References

1. Makoff D. Science agencies get most of what they want, finally. *Science* 2003;299(5610):1160-1161.
2. PHRMA. Research and development. Accessed: May 27, 2003.
3. BIO. Biotechnology industry statistics. Accessed: May 27, 2003.
4. Bodenheimer T. Uneasy alliance: clinical investigators and the pharmaceutical industry. *N Eng J Med* 2000;342(20):1539-1544.
5. PHRMA. Intellectual property. Accessed: May 27, 2003.
6. Goldhammer A. Current issues in clinical research and the development of new pharmaceuticals. *Accountability in Research* 2000;8(4):283-291.
7. Resnik D. Developing drugs for the developing world: an economic, legal, moral and political dilemma. *Developing World Bioethics* 2001;1(1):11-32.
8. Kopelman L. Children as research subjects: a dilemma. *Journal of Medicine and Philosophy* 2000;25(6):745-764.
9. NIH. Questions and answers about the NIH. Available at: http://www.nih.gov/about/FAQ.htm. Accessed: May 27, 2003.
10. Resnik D. Setting biomedical research priorities: justice, science, and public participation. *Kennedy Institute of Ethics Journal* 2001;11(2):181-204.
11. Congress enacted the Orphan Drug Act (ODA) (Public Law 97-414) in 1983 to encourage drug companies to sponsor research on rare diseases and conditions. The ODA, which has been amended several times, gives companies that develop drugs on rare diseases and conditions exclusive

rights to manufacture and sell those drugs for 7 years and also provides tax incentives to companies.
12. Congress enacted the Best Pharmaceuticals for Children Act (21 USC 505) (BPCA) in 1997 to encourage drug companies to test drugs on pediatric populations. The BPCA gives companies an extra 6 months of patent protection for drugs that are tested pediatric populations. Although the BCPA has provided effective economic incentives for the pharmaceutical industry to conduct clinical trials on pediatric populations, the goal of including more children in research raises its own ethical and policy dilemmas. See Kopelman, supra note 8 and Tauer C. Ethical dilemmas on research on children. *Accountability in Research.* 2002;9:127-42
13. American Medical Association. Principles of medical ethics. Accessed: May 27, 2003.

David B. Resnik, JD, PhD is a professor in the medical humanities and director of new programs at The Bioethics Center at The Brody School of Medicine at East Carolina University.