

## Patient Advocates in Research: New Possibilities, New Problems

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I have spent my professional life working in medical schools and law schools—always feeling that I do not quite fit in at either place, but exhilarated by the differences and by the challenges of translation. Focusing one’s teaching and scholarship on bioethics and law is wonderful. The topics grip most people because they concern fundamental moral and personal matters, such as death and dying, reproductive choice, and the pursuit of scientific knowledge. Some of these topics are human embryonic stem cell research, decisions about end-of-life treatment, and access to health care. The focus of this Article, patient advocacy in research, has a somewhat lower profile. This topic is particularly important, however, because it raises issues that touch on many of the topics that keep bioethics in the public eye.

During the 1980s and 1990s, patient advocates became key figures in decisions carrying important ethical and policy implications. Unfortunately, the bioethics community did not pay much attention to this phenomenon. Bioethics scholars *should* have paid attention, however, not only because of the advocates’ growing influence, but also because bioethics is predicated on the premise that public and patient values matter—that physicians, scientists, and government officials should not completely control how medicine and research are practiced. Patient advocates brought to medicine and research the public participation that the bioethics community had been endorsing. As a result, I decided to take a close look at what advocates were doing in research and to consider whether this development was good, bad, or mixed. I eventually wrote a book

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about research advocacy upon which portions of this Article are based.<sup>1</sup>

The first part of this Article supplies background information on advocacy activities. The second part describes five themes in research advocacy. In the third part, I propose three ethical principles to guide research advocacy. I close by discussing three future challenges for patient advocates.

## I. PATIENT ADVOCATES IN RESEARCH

During the 1980s, HIV/AIDS activists became major figures in biomedical research. They helped convince officials at the Food and Drug Administration (FDA) to speed up the drug approval process and to allow patients to use investigational drugs before the full testing process was completed. Scientists began to recognize the valuable contributions that certain HIV/AIDS activists could make to the decision-making process regarding research design, funding, and policy.

Eventually, other patient advocacy organizations became more active in the research arena. Advocates persuaded government officials to ease the rules governing patients' access to investigational medications for a variety of serious, life-threatening conditions. Advocates joined scientific teams in designing, conducting, and interpreting the results of research projects. They served on review panels deciding which research proposals should be funded. Advocates also lobbied Congress for more government funding to support research that could help the advocates' constituents. Additionally, they participated in research ethics reviews at the institutional, state, and federal levels. Their activities raised the public profile of biomedical research, which, in turn, contributed to a media explosion of health research stories.

Advocates' quest for influence changed the politics of biomedical research. In a variety of settings, advocates now challenge government officials and scientists to explain and defend their

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1. REBECCA DRESSER, WHEN SCIENCE OFFERS SALVATION: PATIENT ADVOCACY AND RESEARCH ETHICS (2001).

priorities for research funding and plans for conducting specific studies. In response, scientists worry that advocates focus too much on cures and treatments without recognizing that basic research is the foundation for clinical applications. Researchers also fear that good science is threatened by factors such as the willingness of seriously ill people to “try anything” after conventional therapies have failed. Further, government officials are unsettled by the apparent competition among patient advocacy organizations seeking federal research dollars.

These concerns partly reflect the reluctance of both scientists and government officials to share their power in research decision-making. Yet the concerns are also related to ethical and policy issues raised by patient advocacy. On one hand, patient advocacy could contribute to a biomedical research enterprise that is more attuned to the needs and preferences of the people that the enterprise is designed to assist. Advocates could keep scientists’ personal and professional aims from having too much control over the research agenda. Patient advocacy could also lead to more effective communication of potential study risks and benefits to prospective volunteers. In addition, advocacy offers fresh opportunities for communication between scientists and the public. Increased communication would not only give scientists a valuable insight into the human side of the problems they study, but it would also provide the public with a more realistic picture of how research works and what it can achieve.

On the other hand, positive results are not guaranteed. Genuine communication between scientists and the public is rare. Such communication requires mutual willingness to learn and value the other’s knowledge. Some in the research establishment challenge the use of scarce resources to increase public involvement. Others try to increase involvement cheaply, mostly for public relations purposes. Advocates in policy settings must depart from their usual single-interest lobbying, but their appropriate role in policy activities is unclear. In sum, there are both bright and dark sides to patient advocacy in research.

## II. ADVOCACY THEMES

Five general themes are evident in advocacy activities. First, advocates tend to stress the positive dimensions of biomedical research. In much advocacy communication, there is a failure to clearly distinguish between partially tested experimental interventions and proven medical care. Consistent with this approach, advocates often portray study participation as the way to obtain cutting-edge therapy.

This failure to draw a clear line between investigational interventions and established clinical care affects the perceptions of patients and of the general public. It can promote the therapeutic misconception—a phenomenon that occurs when people do not understand the aims and methodological requirements of biomedical research.<sup>2</sup> The primary purpose of research is to gain knowledge that will improve care for *future* patients, not to deliver treatment tailored for an individual patient. When research and treatment are confused, patients may enroll in research studies without a good understanding of the trade-offs involved. Such patients may have an unrealistic hope for personal benefit without recognizing the risks and uncertainties accompanying their participation in the studies.<sup>3</sup>

Patient advocates may also promote the therapeutic misconception at a broader level. For example, patient advocates often suggest that research can end the suffering and deprivation inflicted by illness. The general message is that with more funding for research, cures are destined to emerge. Although this feel-good message may lift the spirits of people coping with disease and injury, and aid with fundraising, it may also promote public misunderstanding of the research process. There is no question that research *can* lead to health care improvements. Almost always, however, it takes many years and many false starts before effective practical applications become available. But advocates too often downplay this part of research; instead, they equate support for research with support for imminent

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2. The term “therapeutic misconception” was coined by Paul Appelbaum, Loren Roth, and Charles Lidz in 1982. See Paul S. Appelbaum et al., *The Therapeutic Misconception: Informed Consent in Psychiatric Research*, 5 INT’L J. L. & PSYCH. 319, 328-29 (1982).

3. I discuss the therapeutic misconception in further detail in Rebecca Dresser, *The Ubiquity and Utility of the Therapeutic Misconception*, 19 SOC. PHIL. & POL’Y 271 (2002).

improvements in treatment. Advocates present the devotion of more money to research as a way to help patients burdened by disease. Meanwhile, a significant number of those same patients have trouble obtaining existing treatments and services that could extend and improve their lives because they lack adequate health insurance coverage.

Patient advocates are not the only ones to blame for the therapeutic misconception; it has long been encouraged and condoned by many scientists and journalists. But advocates bear some of the responsibility because they occupy a special position of public trust and influence. Meeting their responsibilities to their respective constituents may require a less enthusiastic, more cautionary approach than is common among advocates.

A second theme concerns the way patient advocates evaluate the quality of biomedical research. As one writer put it, patient advocates are “democratizing” biomedical science.<sup>4</sup> This writer observed that advocacy can provoke thoughts “about how science might be different, and under what circumstances difference would mean improvement” in the ways science is currently practiced and evaluated.<sup>5</sup>

In measuring research quality, advocates tend to focus on a project’s ability to benefit patients. When advocates participate in planning studies and setting priorities for funding, they emphasize how the research will affect real people. For example, advocates on a Department of Defense advisory committee, which evaluates proposals seeking funds for breast cancer research, helped establish a custom of starting meetings “with a moment of silence dedicated to a person who is living with or who has died from breast cancer.”<sup>6</sup>

The ultimate purpose of biomedical research is to produce concrete health benefits, and patient advocates emphasize this aim. This emphasis can keep scientists focused on patients’ health needs and counteract competing motivations such as scientific curiosity and desires for career advancement.

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4. Nancy Maull, *Science Under Scrutiny*, HARV. MAG., Mar.-Apr. 1997, at 27.

5. *Id.*

6. INSTITUTE OF MEDICINE, A REVIEW OF THE DEPARTMENT OF DEFENSE’S PROGRAM FOR BREAST CANCER RESEARCH 63 (1997).

However, advocates may be so intent on producing actual health benefits that they end up impeding the research process. For example, advocates may encourage Congress to set aside large portions of research budgets for applied studies, leaving too little for the basic science that underlies much clinical progress. Advocates' quest for treatment improvements can also encourage excessive enthusiasm about novel approaches, which can end up prolonging or preventing the research necessary to determine the safety and effectiveness of those approaches.

An example of the latter problem occurred during the 1990s, when some women's health advocates fought to give women with advanced breast cancer liberal access to high-dose chemotherapy and bone marrow transplantation, a burdensome and costly procedure. These advocates put pressure on insurance companies to pay for the procedure in the absence of solid evidence that it was better than the standard treatment. Unwilling to take the chance of being assigned to the standard therapy group, many women chose not to enroll in randomized clinical trials comparing bone marrow transplantation with standard treatment. These women instead obtained the bone marrow transplantation procedure from physicians outside the research setting. As a result, it took a very long time to enroll enough participants to conduct the trials. When the trials were complete, the data showed that bone marrow transplantation was no better than standard treatment.<sup>7</sup> Women would have been better off if advocates had been more cautious about conveying the procedure's potential benefits. The advocates should have warned women that, like many other experimental interventions, the expensive bone marrow transplantation procedure might turn out to be equivalent, or even inferior, to existing therapies for the disease.

The uneven quality and legitimacy of research representation is the third theme relevant to patient advocacy. Many advocates have a close personal connection to the disease group that they represent because they themselves have, or a close relative has, the disease. Some advocates become "lay experts"<sup>8</sup> with extensive knowledge

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7. See Jonas Bergh, *Where Next With Stem-Cell-Supported High-Dose Therapy for Breast Cancer?*, 355 LANCET 944 (2000).

8. The term is from STEVEN EPSTEIN, *IMPURE SCIENCE: AIDS, ACTIVISM, AND THE*

about the scientific and medical dimensions of the relevant health problem. Others rely solely on knowledge acquired through personal experience with illness. Some advocates receive extensive guidance from their constituents; others appear to take positions based primarily on their own assumptions about what would be best for patients. Some advocates receive funding from drug companies or other sponsors with interests that differ from those of the patients. Individual advocates thus vary in their abilities and qualifications to act as a particular group's representative when making research decisions.

Difficulties in defining the appropriate advocacy constituency may also affect the quality and legitimacy of research representation. Sometimes it is difficult for advocates to figure out which population to serve. For example, should advocates for disease organizations serve current patients, disease survivors, patients' families, and any person at risk, or should they serve only some of these groups? A disease population can be comprised of people with diverse economic, social, and personal interests. An advocate's choice of constituents will therefore affect the practices and policies that the advocate promotes.

The above considerations are also related to fairness, which is the fourth advocacy theme. Advocacy is designed to ensure that the values and preferences of people affected by health problems are at the forefront of research decision-making. But in many research contexts, patients and other constituents have either competing or conflicting interests. People lacking informed and skilled advocate-representatives may lose out when important choices about research are made. Unless advocates are careful, a movement that is aimed at increasing fairness in research decision-making could instead produce substantial unfairness.

Devising a fair advocacy system requires attention to process. When officials and scientists consider only the views of pro-active advocates in their studies and policies, the interests of patients whose advocacy organizations lack the necessary expertise and resources to enter the fray are not taken into account. Thus, to promote genuine

fairness in research decision-making, affirmative outreach and financial support are needed for groups that lack access and representation.

The sheer number of affected groups with a stake in research decisions also creates a fairness issue. There are often practical limits on the number of constituencies that can be directly represented. For example, the National Institutes of Health (NIH) now has a Director's Council of Public Representatives to advise on NIH programs and priorities and to represent the public in various agency activities.<sup>9</sup> But few of the numerous patient-oriented organizations can have an appointed representative, as the Council is comprised of only twenty members. This practical constraint creates a need to devise fair procedures for selecting Council members and methods for obtaining the views of groups that are not directly represented on the Council.

Fairness also depends on officials' and researchers' responses to patient advocacy. Fairness is *not* promoted when officials and researchers smile and appear to listen, but then ignore patient advocates. A fair decision-making process requires features ensuring that advocates' views are taken seriously. Such features include required explanations of the reasons for research decisions and, in some situations, a chance for advocates to present arguments for revising those decisions.

A fifth theme concerns the relationship of patient advocates with people whose academic and professional focus is bioethics. Advocates and bioethicists have some common goals. For example, both groups believe it is essential to bring the values and concerns of ordinary people into research ethics and policy debates. Yet there is discord in the relationship as well. While advocacy stresses the benefits attainable through research, conventional ethics put as much or more emphasis on the harms that can result. Ethicists are likely to see certain advocacy activities as disturbingly promotional, and advocates often see ethicists as paternalistic and overprotective.

These differences may be part of the reason that advocates and bioethicists have had little to do with each other. Does this separation make sense? If I thought it did, I would not have written a book about

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9. Notice of Establishment, 63 Fed. Reg. 63,938 (Nov. 9, 1998).

advocacy and research ethics. I think that the two groups could benefit immensely from increased interaction. With cooperation, the two groups could effectively promote their mutual aims and help each other examine the ethical implications of their work. Debates over conflicting views would benefit the two groups as well because each would come away with more reasoned positions and an improved understanding of their remaining disagreements.

### III. ETHICAL PRINCIPLES FOR PATIENT ADVOCACY

Patient advocates are now influential and trusted participants in debates over research projects, policies, and funding priorities. Advocates should be aware of their ethical responsibilities in representing patients and lobbying for government and private research support. In an effort to encourage reflection on the ethics of patient advocacy, I offer three principles to guide patient advocates in research.

First, advocates should be accurate and realistic when communicating with their constituents about research. Advocates should recognize a fiduciary responsibility to be honest about the uncertainties inherent in biomedical research. They should emphasize that scientific progress occurs slowly and incrementally, and that initially promising developments often fall short when subjected to further study. When advocates disregard this principle, patients and their families, as well as the broader public, are more inclined to entertain unfounded hope.

Also, when advocates express undue optimism about research, they encourage others to give that research a disproportionate priority in terms of policies and funding. Research advocacy should be accompanied by advocacy aimed at increasing patients' access to existing treatment and services that could extend and improve their lives. Advocates must recognize that even the most promising research typically offers little help to patients today. Advocates should promote efforts to furnish patients with the therapies, home care, social services, and palliative care that could confer immediate health benefits.

A second ethical principle instructs advocates to consider the diversity of their constituents. Advocates have a tendency to promote

policies that favor just one segment of their constituents—well-educated and well-insured patients, along with their families. This constituent group is able to take advantage of policies designed to increase patients' access to clinical trials and unapproved interventions outside of trials, including policies requiring insurance coverage for patients in clinical trials and websites describing new trials seeking volunteers.

But not all advocacy constituents have sufficient education and resources to navigate the research world on their own. For instance, some lack Internet access or skills. These individuals cannot take advantage of websites presenting information on clinical trials. Additionally, some constituents lack insurance coverage. These individuals have problems getting good standard care; insurance coverage for clinical trials does not benefit them.

This diversity creates a need for advocates to communicate directly with constituents in different economic and educational situations. Consultation with a broad range of constituents will allow advocates to formulate decisions and policies that take into account the interests of all of their constituents.

A third ethical principle holds that advocates should reject parochialism—the focus on a single disease or population group—in funding and policy work. Strong partisanship for one group is unfair when not everyone affected by a decision is equally represented. Advocates should recognize that patients and others outside of their usual disease constituencies have morally significant interests in many research decisions. Advocates should thus promote processes that allow broad and fair representation of disease organizations when policies and funding priorities are debated. Advocates should also make efforts to work collaboratively, rather than competitively, with other organizations.

#### IV. FUTURE CHALLENGES FOR PATIENT ADVOCACY IN RESEARCH

Three challenges face patient advocates in their future research activities. The first challenge is determining whether HIV/AIDS activism should continue to serve as the primary model for research advocacy. Certain features of HIV/AIDS advocacy are positive and should be emulated by other advocacy organizations. For example,

many HIV/AIDS advocates have acquired extensive medical and scientific knowledge about the disease. This strategy has increased HIV/AIDS advocates' credibility and effectiveness with scientists and policymakers. Another positive characteristic of HIV/AIDS advocates is their grassroots approach to communicating with patients. This approach keeps advocates aware of their constituents' ongoing concerns and helps advocates avoid becoming co-opted by the research establishment.

Certain features of HIV/AIDS advocacy are less suited to other advocacy organizations, however. For example, the central aim of HIV/AIDS advocates has been to promote research that would lead to a cure for the disease. But advocates for people with conditions such as Parkinson's disease and Alzheimer's disease have constituents suffering from chronic, degenerative diseases that occur later in life. Research on these conditions is much less likely to yield a cure that benefits current patients than is research on HIV/AIDS, an infectious disease that primarily strikes young and middle-aged people.

Another problem is that some HIV/AIDS activists have too eagerly embraced disease theories and treatments that lack a solid scientific basis. Their behavior has encouraged patients to take ineffective and sometimes harmful underground drugs. This precedent should not dissuade advocates from scrutinizing mainstream science. Rather, it should stand as an illustration of the dangers posed by wishful thinking and oversimplification in advocacy work.

The second challenge for advocates is to develop adequate responses to the rise of industry-sponsored clinical research. Advocates in today's "market economy for research"<sup>10</sup> should alert patients to the possibility that industry research sponsors may offer financial rewards to physicians who enroll people in trials. Advocates should insist on adequate reviews of the ethics of research proposals and warn industry sponsors that they will not permit deviations from accepted ethical standards. Advocacy organizations should also formulate conflict of interest policies to govern their relationships with drug companies and other commercial enterprises, because

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10. Paul T. Kefalides, *Research On Humans Faces Scrutiny: New Policies Adopted*, 132 ANNALS INTERNAL MED. 513 (2000).

industry research sponsors are increasingly offering financial support to advocacy organizations that agree to help them with study recruitment and related activities.

The third challenge facing patient advocates is to formulate positions on xenotransplantation,<sup>11</sup> human embryonic stem cell research, and other novel research areas. Meeting this challenge will require advocates to understand new and complex scientific information. It will also require them to address situations in which patients' interests in promoting research and advancing knowledge may conflict with other societal interests, such as religious and moral concerns about the protection of early human life, or public health concerns about infectious diseases that animal organ transplants might produce in humans. In the future, patient advocates may encounter previously unseen types of opposition. To continue as effective advocates, they will have to develop persuasive and responsible positions in response to this opposition.

In writing about this topic, my goal is to help advocates, researchers, public officials, and bioethicists think systematically and creatively about how to promote ethical and effective advocate involvement in research decision-making. I also hope to help patients and the public gain an understanding of what to expect from biomedical research and from patient advocates, scientists, government officials, and science journalists. Furthermore, I hope to give scholars an insight into the advocacy phenomenon and build a foundation for future work. I consider my analysis only a first take on patient advocacy in research and look forward to hearing what others have to say about this fascinating feature of the contemporary research landscape.

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11. Xenotransplantation is the transplantation of an organ from an individual belonging to one species into the body of an individual belonging to a different species. The goal of this research is to extend the lives of humans through transplantation of non-human hearts, kidneys, livers, and other vital organs. *See* INSTITUTE OF MEDICINE, XENOTRANSPLANTATION: SCIENCE, ETHICS, AND PUBLIC POLICY (1996).