

# A Third Seat at the Table: *An Insider's Perspective on Patient Representatives*

BY DUANE ROTH

As the nation takes a fresh look at all aspects of medical care, advances in the health sciences—from genetic sequencing to stem cell technology—may give us opportunities to make health care better, faster, and more cost-effective. But first we must transform our approach to health care innovation, particularly in the regulation of new products that may signal clinical breakthroughs.

These issues intersect at the table where federal agencies, particularly the Food and Drug Administration, and innovative product development companies negotiate the regulatory approval process. At present, both parties are overly constrained. The agencies face too many “but what if?” disincentives: potential and unknown safety risks and the specter of adverse publicity. The sponsors are wary of investing time and resources in the absence of a defined regulatory pathway. Too often, inaction seems the safest course.

But for the people most directly impacted by disease, inaction is irresponsible, even unethical. Patients and their families have the greatest stake in the approval process. Their lives, and their quality of life, hang in the balance. They are in the best position to weigh actual risks and benefits. It is time to give patients, through carefully selected representatives, a seat at the regulatory approval table. If they serve as mediators, not just advisers, they can help regulators and companies identify new pathways for fostering innovation, building public trust, cutting costs, and addressing quality-of-life issues.

Three decades of experience demonstrate that informed and dedicated patient representatives can break through

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development impasses. And since 2005, designated “patient advocates” on the governing board of the California Institute for Regenerative Medicine (CIRM), which I have served on for the last four years, have shown that patients are uniquely qualified and motivated to help decide how medical products should be delivered responsibly and expeditiously.

## AIDS and the FDA Sea Change

The concept of the patient representative came to prominence in the 1980s AIDS epidemic, which galvanized patient communities to unprecedented levels of scientific and political involvement. In the San Francisco Bay Area, patient advocates organized their own studies of HIV/AIDS drugs with local doctors and volunteer subjects. They conferred with pharmaceutical manufacturers and exerted pressure on the FDA and the National Institutes of Health. Their efforts helped bring about a sea change in AIDS clinical trials and drug regulation. The FDA revamped its approval criteria, and community advisory boards began working with institutions that received NIH AIDS-related grants.

Starting in the 1990s, the FDA invited patient representatives to serve on advisory committees that review products for life-threatening diseases. But patient members typically occupy less than 10 percent of the slots on those committees, and they continually struggle to exert real influence on product decisions.

After the FDA approved the multiple sclerosis drug Tysabri in 2004, manufacturers withdrew it when some patients in clinical trials developed a rare brain disease. Prolonged efforts by MS patient groups helped bring about the 2006 FDA reintroduction of the drug and a new government-industry program to educate patients about risks. As John Richert of the National Multiple Sclerosis Society said, “We just have to learn . . . how to balance those risks and benefits for each individual person who needs to be treated for their MS.”<sup>1</sup>

In 2007, an FDA advisory committee studying the prostate cancer drug Provenge endorsed its safety by a vote of seventeen to zero and its efficacy by a vote of thirteen to four. But the agency felt that it needed more data on safety and efficacy. Additional clinical studies resulted in several years of delay before approval was granted this past April. While the FDA may have been exercising due diligence in asking for further study, the delay caused an outcry from the national cancer patient community and was a key factor in the bipartisan introduction of the Access, Compassion, Care, and Ethics for Seriously Ill Patients (ACCESS) Act, legislation that is still pending in Congress.

## The CIRM Experience

There is a longstanding ethical argument that patients afflicted with illnesses should be able to participate in the search for treatments and cures. I have gained new respect for that principle during my service on the CIRM governing board, known as the Independent Citizens’ Oversight Com-

mittee, where I currently serve as its vice chairman representing industry.

The twenty-nine members of the ICOC are distinguished Californians who represent academic science, industry, and patients. The ICOC makes decisions about research funding, clinical applications of emerging products, and legislative proposals. The committee also is responsible for shepherding new projects to advance stem cell research. Ten ICOC members, in addition to the chair and vice chair—more than one-third of the total—are “patient advocates” who speak for ten major disease groups with a stake in stem cell science. They are respected leaders from diverse professions in the public and private sectors. They have had direct personal experience with life-threatening illness as patients, survivors, or caregivers. They have no allegiance to and they do not accept funding from any interest groups. As with all ICOC members, their formal charge from CIRM is to “represent the expertise and passion of the people of California.”

In my estimation, the patient advocate members have a keen grasp of the issues the ICOC must decide, including governing policies and procedures, scientific data, and intellectual property rights. They are acutely aware that many basic biological mechanisms often yield useful information across a wide spectrum of diseases, and therefore, they often act in concert with advocates from other disease areas to explore common pathways for broader overall benefit. Perhaps most importantly, patient advocates understand and articulate better than anyone that seriously ill patients will accept risks associated with new products in exchange for benefits that might not happen immediately. As Jeff Sheehy, the ICOC patient advocate for HIV/AIDS, explained it in a recent conversation with me, “There may be high risks, and the reward may be far down the road. But in many diseases, that’s critical, and for many patients, that’s valuable.”<sup>2</sup>

These representatives speak with authority about patients’ experiences with existing therapies and their willingness to tolerate side effects from new therapies. “Those aren’t always scientific decisions because they often aren’t based on scientific evaluation,” said Sheehy. “These are ultimately decisions that patients make in consultation with their families and their health care providers. And after patients and their families and providers are fully informed of the real or potential consequences, regulators should allow them the autonomy to make their own decisions.”

And ICOC patient advocates bring trust and accountability to discussions of new products because they can raise questions about any aspect of development, including regulators’ intransigence and companies’ inflated claims. “We question the grants in a respectful way, and we almost always defer to the scientists,” said Sherry Lansing, the ICOC patient advocate for cancer. “But because we are the face of the disease, we convey the urgency of getting products to clinical trials.”

Since I joined the ICOC in 2006, I have seen numerous examples of how patient advocates clarify our deliberations and guide us to render judicious decisions. Two examples offer striking lessons in the unique value of patient mediators.

## Intellectual Property Management

One of our first policy decisions for the ICOC involved management of the intellectual property derived from CIRM grants. This was assigned to an Intellectual Property Task Force, a subcommittee of the ICOC led by Ed Penhoet, a biochemist who had served as chief executive officer of a biotech company, Chiron Corporation, as dean of the University of California at Berkeley School of Public Health, and as president of the Gordon and Betty Moore Foundation. I was a member of his task force, which held more than twenty public meetings and seventeen rounds of public comment.

The California Stem Cell Research and Cures Initiative that established CIRM mandated an intellectual property policy that would achieve three goals: (1) assure that the state of California would benefit from patents, royalties, and licenses; (2) enable essential research to advance to product development without obstacles that might arise from IP agreements; and (3) disseminate scientific data and advanced knowledge through timely publications.

Public opinion ran the gamut. Some stakeholders, including consumer watchdog groups, favored high royalties and quick payback to the state. Others, including industry trade groups, wanted no payback at all, in keeping with the policy of the NIH. Some wanted CIRM to oversee the price of new products; others wanted CIRM to leave that completely open to the market. Publication and data sharing were supported by the scientific community and consumer groups but opposed by industry.

The ten-member IP Task Force included three patient advocate members. A reading of the task force meeting transcripts shows that, amid this swirl of perspectives, the patient advocates worked toward a compromise that would not have been reached by scientists and industry representatives alone. With a focus on the desire for access to new therapies, the patient advocates argued persuasively that such products would not come to market if industry had to face a low return on investment and poorly defined future obligations. With equal passion, they agreed with scientists and consumer groups, in the face of industry apprehension, that widespread and timely scientific data dissemination was imperative.

The resulting policy has been accepted by CIRM grantees, industry, and consumer groups, and it has even been praised by public policy watchdog groups. In a letter to Penhoet, John M. Simpson, stem cell project director for the Foundation for Taxpayer and Consumer Rights, hailed the IP task force as a “perfect model for soliciting and considering input from all stakeholders.”<sup>3</sup>

## “Biosimilars” and Exclusivity Periods

The second example of the value of patient mediators involves the Patient Protection and Affordable Care Act passed by Congress in January. A provision in the bill, the “Biologics Price Competition and Innovation Act,” sought to

establish a regulatory pathway for generic biologicals, or “bi-osimilars,” something the biotech industry has long opposed.

At an ICOC meeting, I suggested that CIRM should consider taking a position against this legislative provision. I argued that generic biologicals would create a disincentive for investment in early-stage, high-risk therapeutics including stem cells. At the time, there were two competing legislative proposals, one for a seven-year innovator product “market and data exclusivity” period, and the other for a twelve-year exclusivity period.

The initial discussion at the board meeting did not produce a consensus. Industry members either believed that all follow-on biosimilars should continue to provide a full battery of preclinical and clinical trial data prior to FDA approval, or they wanted a twelve-year exclusivity period before a competitive product could rely on the innovators’ data for approval.

Scientist and patient advocate members all supported a pathway to biosimilars, but they were divided on implementation. Some favored the shorter exclusivity window; others, the longer. A few did not think CIRM should even be involved in the issue.

Eventually, the patient advocates tipped the scales in favor of the longer, twelve-year period. They concluded that the need for significant investment and the greater financial risk in unproven therapies outweighed the need for a shorter path for lower-cost “biosimilars.” Their influence led the board to a unanimous decision in support of the twelve-year period.

Senator Dianne Feinstein, a member of the Senate Health Committee, followed up with a compelling letter that echoed the board’s concerns and added her own support for the longer exclusivity period. The CIRM position has since been cited as a pivotal event in the debate leading up to the final 2010 health care legislation.

### **Moving Forward: A First Step**

If patient representatives participate in negotiations as mediators, they can serve to balance risks and benefits and determine the appropriateness of any approval plan. Think of this approach as engineering out biases and building in trust. Patient mediators will be a catalyst for making real progress on urgently needed products. And perhaps most importantly, they will provide support for regulators and sponsors when

unforeseen complications arise that could spark political pushback.

Traditional patient advocacy has been criticized as inherently biased because patients may be so desperate for cures that they will disregard most or all risks. The current system has safeguards to preclude such bias: individual patients and their doctors, institutional review boards at each clinical site, and the FDA Advisory Board at the national level. The CIRM model offers another safeguard: collaborative decisions that are made after open and vigorous debate. Patient advocates engage fully with scientists, consumer representatives, and industry leaders. Our board deliberations are made public.

We are keenly aware that our primary stakeholders—Californians—scrutinize our work, and we welcome that scrutiny.

How to implement that? The FDA could take a first step by agreeing to a pilot project in which patient mediators would be invited into negotiations for a specific innovative new product. The patient mediators would be best represented as a team whose qualifications would include a direct relationship to the disease area either as a patient or as an immediate family caregiver. And collectively, team members would need knowledge of the regulatory process, statistical analysis, clinical development, and the manufacturing process.

If the FDA is unable to legally implement this process—or unwilling—patient mediators could be legislated into the process of new product regulation, with the FDA retaining final authority for the approval process.

The time is right and the stage has been set for a national model that incorporates patient mediators into the approval process. The costs would be negligible, and the payoffs in therapeutic efficacy, procedural efficiency, and public confidence could be enormous.

### **Acknowledgments**

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1. The National Multiple Sclerosis Society, *MS Learn Online: Tysabri, What You Need to Know, Part 1*, <http://www.nationalmssociety.org/multimedia-library/webcasts--podcasts/tysabri/index.aspx>.
2. All quotes from ICOC patient advocate members are from recent interviews with the author.
3. Letter cited in *The California Stem Cell Report*, September 25, 2006, [http://californiastemcellreport.blogspot.com/2006/09/watchdog-group-praises-cirm-ip-task\\_25.html](http://californiastemcellreport.blogspot.com/2006/09/watchdog-group-praises-cirm-ip-task_25.html).

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