

Advocates deserve room at the decision-making table

Jeff Sheehy

Patient advocates are often dismissed by the scientific establishment for focusing too much on cures and treatments at the expense of basic research. But advocates help create a biomedical research enterprise that is more attuned to the needs and preferences of the public—the very people who ultimately support and are meant to benefit from the enterprise. As such, scientists and government officials would be wise to heed patient advocates' advice.



Bill Berry

On a November afternoon in 2004, my phone rang, with California's Senate leader John Burton on the line. "I'm putting you on this board, OK?" he asked. I said "sure," barely understanding what he was talking about. Thus, I became a patient advocate member of the Independent Citizens' Oversight Committee, the governing board of the California Institute of Regenerative Medicine (CIRM)—the \$3 billion stem cell agency set up a few weeks earlier through a ballot initiative approved by 59% of California voters.

Unlike most granting bodies, such as the US National Institutes of Health (NIH) or similar agencies elsewhere, CIRM's board was mandated to include 12 patient advocates, including the chair and vice-chair, who represent a wide range of diseases. And, unlike the other members of the 29-person board, which includes scientists, medical school deans and biotech executives, the advocates also have slots on the agency's three working groups, including the Grants Working Group that serves as the peer-review committee for CIRM.

All research proposals go through this working group, of which I am vice-chair. Whereas the 15 non-Californian scientist members score the proposals (aided by a bevy of additional specialists), the seven advocate members in this working group join in the voting process to determine funding recommendations.

The presence of vocal, engaged patient advocates has added an indispensable dimension to the proceedings. In measuring research quality, advocates tend to focus on a project's ability to benefit people—not just drive scientific curiosity—which keeps even basic biomedical research grounded in its ability to produce concrete health benefits.

For example, as a person living with HIV, I have challenged the understandable, but patronizing, assumption that life-saving combination antiretroviral therapies are sufficient for patients with HIV. I have described to the CIRM board and staff scientists the real side effects of these medications, and humanized the HIV-and-aging syndrome that includes increased rates of heart disease, non-HIV-related cancers and neurological deficits. When a reviewer opines that he does not deem a proposal worth funding because he doesn't think anyone with HIV will consider participating in clinical trials of an experimental treatment, I answer that I would, and my voice has fundamentally changed the discussion.

Stem cell approaches for HIV treatment in particular have had to fight strong headwinds. In 2008, after doctors in Berlin reported that a stem cell transplant resulted in the first functional cure of an HIV patient—meaning control of HIV without requiring therapy—Anthony Fauci, head of the US National Institute of Allergy and Infectious Diseases, told the *New York Times* that "it's very nice, and it's not even surprising. But it's just off the table of practicality."

But even though one of the most influential scientists in the country dismissed the study's relevance, patient advocates like me instantly

recognized the research's potential. As a result, last year the highest-scored application for a CIRM disease team grant—up to \$20 million, with the goal of a clinical trial within four years—was an attempt to replicate the results in the 'Berlin patient' using gene therapy techniques and hematopoietic stem cells. We also funded a similar application that was the fifth highest scored in the round (*Nat. Med.* **16**, 731, 2010).

Patient advocates serving on CIRM's working groups are not only moving projects forward that have languished as a result of prevailing dogmas at other funding agencies but also playing major parts in resolving ethical issues. Under the leadership of film executive Sherry Lansing, a cancer patient advocate and co-chair of our Standards Working Group, CIRM developed ethical standards regarding thorny questions such as egg donation and informed consent for embryo donation after open meetings with full public participation.

The NIH does not completely shut out patient advocacy organizations, mind you. Through the Director's Council of Public Representatives, which advises on agency activities, the public has a voice at the table. And some patient advocates are invited to serve on individual institutes' advisory councils where they, along with scientists and clinicians, make funding recommendations and offer other advice to the relevant institutes' directors. But these arrangements rely on the directors' willingness to listen and be inclusive. It is quite different to have a formal decision-making role written into the statute.

Many foundations also empower patient advocates with substantial influence. Some, like the Juvenile Diabetes Research Foundation, are almost entirely patient driven. But few of these entities are dealing with the amount of funds available at CIRM, and they're ultimately accountable to donors, not to public taxpayers at large. What's more, unlike single disease-specific foundations, CIRM is made up of patient advocates from a wide spectrum of diseases and conditions who work together to advance therapies across the board. And contrary to critics' assertions, these advocates have not narrowly focused on their own diseases, but have uniformly advocated for the best approaches for moving basic research towards the clinic. They support each other.

I have experienced trepidation from some scientists confronted with the CIRM model of active patient advocate involvement. To some degree, this stems from lack of experience working with advocates; in other instances, one might chalk it up to ego. But it's undeniable that CIRM and its billions of dollars would not exist without the work of patients in convincing the people of California to support the venture. Furthermore, I firmly believe that the CIRM vision of patient advocate inclusion will prove to accelerate the path to cures.

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