Perspectives On CIRM/Prop 71
From The Patient Advocacy Community

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Patient Advocate Member - CIRM Governing Board

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Model Scientific And Patient Advocate Partnerships Advancing Therapies

Major examples:

- **Polio Vaccine**
  - FDR (highly visible patient advocate as polio patient)
  - March of Dimes, a novel funding and community mobilization mechanism established 1938
  - Leadership shared between Basil O’Conner and Dr. Jonas Salk and vaccine success achieved in 1955

- **HIV/AIDS**
  - Huge burden of stigma
  - Cohesive, militant response rewrites rules for clinical trials and drug approvals
  - 1996, combination ARV emerges, 15 years after 1st cases

- **Stem Cell Advocacy/Proposition 71**
  - Latest iteration of patient advocacy and scientific community collaboration
  - Potential new model engaging multiple diseases and conditions advocates in a broad coalition with researchers
Proposition 71 – A Paradigm Change In Governance: The Role Of Patient Advocates

Patient Advocates’ Roles

- Written into statute: (12 of 29 seats on CIRM’s Governing Board are reserved for designated Patient Advocates)
  - Chair & Vice Chair must have patient advocate credentials
  - Ten Patient Advocates

- Working Groups central to CIRM’s functioning include patient advocate board members and external experts

- Patient Advocates share Working Group leadership roles
  - Grants Review: Jeff Sheehy & Joan Samuelson (Co-Vice Chairs)
  - Standards: Sherry Lansing (Co-Chair)
  - Facilities: David Serrano-Seewell (Vice-Chair)

- Service on ICOC Subcommittees and Task Forces

CIRM Public Meetings:
- ICOC meetings: 77
- Subcommittee/Task Force meetings: 122
- Standards working group meetings: 25
- Grants working group meetings: 30
- Facilities working group meetings: 19
Patient Advocates Representing CIRM

Current
• Marcy Feit, R.N., MSN – Patient Advocate – Type II Diabetes
• Leeza Gibbons – Patient Advocate – Alzheimer’s Disease
• Sherry Lansing – Patient Advocate – Cancer
• Francisco Prieto, M.D. – Patient Advocate – Type I Diabetes
• Robert Quint, M.D., FSCAI – Patient Advocate – Heart Disease
• Joan Samuelson, J.D. – Patient Advocate – Parkinson’s Disease
• David Serrano Sewell, J.D. – Patient Advocate MS/ALS
• Jeff Sheehy – Patient Advocate – HIV/AIDS
• Jonathan Shestack – Patient Advocate – Mental Health
• Os Steward, Ph.D. – Patient Advocate – Spinal Cord Injury

Former
• Phylis Preciado – Patient Advocate – Type II Diabetes
• Leon Thal, M.D. – Patient Advocate – Alzheimer’s Disease
• Janet Wright, M.D. – Patient Advocate – Heart Disease
Proposition 71

Disease and Patient Advocacy Organization Endorsements
(Partial List of over 70 Organizations)

AIDS Research Alliance
ALS Association
Alliance for Aging Research
Alzheimer’s Association, CA Council
American Diabetes Association
American Lung Association of CA
Asthma & Allergy Foundation of America
California Arthritis Foundation Council
Cancer Research and Prevention Foundation
Christopher Reeve Foundation
Coalition for the Advancement of Medical Research (CAMR)
Cure Autism Now Foundation
Cystic Fibrosis Research, Inc.
Elizabeth Glaser Pediatric AIDS Foundation
International Society for Stem Cell Research Foundation
Juvenile Diabetes Research Foundation
Late Onset Tay-Sachs Foundation
Leeza Gibbons Memory Foundation
The Leukemia and Lymphoma Society
Lupus Foundation of America
Michael J. Fox Foundation for Parkinson's Research
National Brain Tumor Foundation
National Medical Association
National Multiple Sclerosis Society
Parkinson’s Action Network
Parkinson’s Disease Foundation
Project ALS
Prostate Cancer Foundation
Research!America
San Francisco AIDS Foundation
Sickle Cell Disease Foundation of California
Stem Cell Action Network
Women’s Cancer Research Fund
Advocates Deserve Room At The Decision-Making Table

Advocates deserve room at the decision-making table

Jeff Sheehy

Patient advocates are often dismissed by the scientific establishment for focusing too much on cares and treatments at the expense of basic research. But advocates help generate 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.

On a November afternoon in 2004, my phone rang, with California Senate leader John Burton on the line. “I’m putting you on this board,” he said. I asked “what” board he was referring to. “This one,” he said, referring to the California Institute for Regenerative Medicine (CIRM) —the $3 billion stem cell agency approved by 94% 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 32 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, patient advocates also have seats 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 non-California scientist members score the proposals (aided by a bevy of additional specialists), the seven advocate members in the group are able to join the voting process to determine funding recommendations.

The presence of vocal, engaged patient advocates has added an indispensable dimension to the process. In reviewing research quality, advocates tend to focus on a proposal’s ability to benefit patients and the community—at any cost—rather than basic biomedical research grounded in its ability to produce concrete health benefits.

For example, as a person living with HIV, I have championed the understandability, but not necessarily the efficacy, of the DMPA vaccine for HIV. Because the CIRM board and staff scientists see the real-world effects of these medications, they understand the disease and the need for a vaccine.

They do not judge the research worth funding because they don’t think anyone with HIV will enter participating in clinical trials of an experimental treatment. I favor that approach, and my voice has fundamentally changed the discussion.

Stem cell approaches for HIV treatment in particular have had to fight strong headwinds. In 2006, 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 part of the table of practice.”

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 potential. As a result, last year the highest-scored application for a CIRM disease team grant—up to $30 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 bone-marrow 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 merely moving projects forward that have languished as a result of previous decisions at other funding agencies but also playing major parts in resolving ethical issues. Under the leadership of executive Sherry Lansing, a cancer patient advocate and co-chair of our Standards Working Group, CIRM developed ethical standards regarding therapy 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 seat at the table. And some patient advocates are invited to serve on individual institute advisory councils where they, along with scientists and clinicians, make funding recommendations and recruit peer reviewers to advise the institute’s 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 informal 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 are 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 are not narrowly focused on their own diseases, but have uniformly advocated for the best approach for moving basic research towards the clinic. They support each other.

I have experienced frustration from some scientists confronted with the CIRM model of active patient advocate involvement. To some degree, these stem from lack of experience working with advocates in other instances, one might fault it up on age. But it is 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-driven activation of patient advocates will prove to accelerate the path to cure.

Jeff Sheehy is director of communications at the University of California-San Francisco AIDS Research Institute and chair of the CIRM Governing Board. Science subcommittee.

• Partnership with patient advocates improves processes and outcomes

• Real seats at the decision-making table and tangible influence, a vital part of the Proposition 71 compact between the research community and the advocacy community
Prop 71: An Improvable Model for Reengineering the Medical Research Process?

- Serious and Worsening Inefficiencies in the Therapy Development Process
  - Andy Grove’s Presentation at World Stem Cell Summit 2011
    - Crisis in Translational Medicine
    - New therapies taking longer and are more expensive with a higher attrition rate
    - Cells and genes will be harder and more expensive to develop than small molecules/pills
    - Clinical trial/therapy development process archaic, inefficient and flawed
    - FDA needs to reconsider regulatory pathways and processes, especially in era of cells, genes and personalized medicine

- New Business Models and Therapy Delivery Systems for Cells/Genes Likely Required

- In Challenging Public Funding Environments, Creating and Sustaining Support for Biomedical Research Funding is a Major Issue

- Patient Advocates Across Diseases and Conditions at the Table in Meaningful Partnership with the Research Community – A Dynamic Catalyst for Change (i.e., Prop. 71 model on a national scale)?
Thank You

For more information: www.cirm.ca.gov