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New California stem cell chief stresses speed and efficiency in search for treatments

Kenneth P. Serbin
University of San Diego

At Risk for Huntington's Disease

HD is a genetically caused brain disorder that causes uncontrollable bodily movements and robs people's ability to walk, talk, eat, and think. The final result is a slow, ugly death. Children of parents with HD have a 50-50 chance of inheriting the disease. There is no cure or treatment.

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New California stem cell chief stresses speed and efficiency in search for treatments

A major hope of those facing Huntington's disease (and numerous other diseases) resides in stem cell research.

The new president and CEO of the California Institute for Regenerative Medicine (CIRM), transferring from the pharmaceutical industry, has assumed the helm of the $3 billion organization stressing efficiency, including a pledge to prioritize speedier development of treatments for the many diseases falling within the agency's scope.

"What I promise I will do is to bring stem cell therapies and treatments to the patients that need them," C. Randal Mills, Ph.D., chosen to run CIRM by its board of directors on April 30, said in San Diego on June 24 at the third of three "Meet the New CIRM President" events. "That is quite sincerely what I have done my entire career, and the only thing I care about and the only reason I came to CIRM."

Dr. Mills was introduced by CIRM board chair Jonathan Thomas, J.D., Ph.D. The meeting took place in conjunction with the 2014 BIO International Convention, June 23-26, which showcased the work of leading biotech firms and featured a keynote speech by British business magnate Sir Richard Branson and a moderated Q & A with former Secretary of State and potential 2016 presidential candidate Hillary Rodham Clinton. The convention attracted more than 15,000 participants from all 50 states and 70 countries.

Dr. Mills outlined four questions he said will guide him in decision-making at CIRM.

First, he said, "is whatever we're doing speeding up a treatment reaching a patient?"

Secondly, will CIRM's activities increase the likelihood of a treatment reaching a patient? There are many "valleys of death," or dead ends, in stem cell research, Dr. Mills noted.

Third, is CIRM meeting an unmet medical need, as opposed to a condition already successfully dealt with by other medical means?

Fourth, is CIRM doing all this efficiently?

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Randy Mills speaks to disease advocates and stem cell industry representatives in San Diego (photo by Gene Veritas).

Taking care of patients

Dr. Mills said his patient-oriented outlook started during his undergraduate studies in microbiology and cell studies at the University of Florida, in Gainesville.

“During that time I worked as a medic in the emergency room,” he told the audience. “I saw and dealt with a lot of patients and got a pretty good sense of what patient care was like and delivery was like.”

Dr. Mills obtained his Ph.D. in drug development, also at the University of Florida. After that, he worked for the university as a specialist in orthopedic transplants. With a partner, Jamie Grooms, he started a company within the university specializing in spinal fusion, one of the most common of orthopedic procedures.

In 1995, the two “spun out” the company from the university, calling it University of Florida Tissue Bank. That year the company had $1 million in revenues, with only six employees. Five years later, when the firm went public, it had 550 employees and annual revenues of $120 million.

“More importantly, (we were) producing regenerative medicine solutions for patients all across the United States on the scale of hundreds of thousands of implants, and better implants, a year,” Dr. Mills explained.

“It was during that time that I really learned a lesson. And the key lesson is: if you take care of patients, then your business is going to follow. If you don’t take care of the patients, there is nothing you can do in order to get your business to come along.”

Randy Mills (Osiris photo)

Key achievements at Osiris

In 2004, at the age of 32, Dr. Mills was recruited to become the president and CEO of Osiris Therapeutics, Inc., a Columbia, Md.-based company that commercialized the world's first stem cell product, Osteocel, for bone regeneration. According to Mills, that product has brought a total of $1.5 billion in revenue to Osiris.

Under his leadership, in May 2012 Osiris received approval to market the world's first systemically infused stem cell drug, Prochymal, which it developed to combat pediatric acute graft-versus-host disease. (It was approved in Canada but is also available in the U.S.; click here to read more.)

This condition occurs in patients receiving bone marrow transplants that reject the person and attack the body.

"Patients will literally peel out of their skin," Dr. Mills said, describing the horrors of the condition. Patients with the condition have a life expectancy of only 87 days, he added.

With Prochymal, patients got better two-thirds of the time, he said.

Dr. Mills attributed Osiris's success to its intense focus on patients.

"The board room is covered with pictures of our patients," he said.

"That's my mission with CIRM," he continued. "We're going to focus on the patients, and everything else is going to come along. If you get a sense of urgency from me, it's because, if a life expectancy of a disease is 87 days, missing a month or two months or three months are actually real patients dying."

Putting criticisms of CIRM in perspective

The stem cell board's selection of a new CEO with long experience in the

drug industry takes place a decade after California voters created CIRM by approving Proposition 71, the California Stem Cell Research and Cures Act.

According to CIRM's statistics, so far four clinical trials directly funded by the organization have taken place – including an observational study of Huntington's disease patients at the University of California, Davis, the basis for a potential CIRM-supported treatment trial envisioned by Dr. Vicki Wheelock and Dr. Jan Nolta (click here to read more).

Six additional trials for different conditions are based on "discoveries made by our grantees when they were carrying out CIRM-funded research," CIRM reports (click here to read more).

According to Kevin McCormack, CIRM's senior director for public communications and patient advocate outreach, five more directly funded trials for various diseases will start by the end of 2014.

CIRM's efforts have not yet produced a drug, although one or more treatments could arise from the clinical trials.

Some in California have criticized CIRM's performance. The *San Francisco Chronicle*, for instance, editorialized that CIRM "hasn't lived up to its hype" and has compiled a "decidedly mixed" record, although it recognized that California voters had "outsize expectations when they passed Prop. 71."

The *Chronicle* further noted that "it's been a struggle to get the agency to use the best organizational practices. In 2012, a blue-ribbon committee of the National Academy of Sciences released a report after a yearlong review that found conflicts of interest on the CIRM board that threatened to 'undermine respect for its decisions.' It also found significant flaws in the agency's grant-approval process."

The editorial added: "Progress on stem cell research has been significant – but it's been the progress of the tortoise rather than the hare."

In general, news coverage of CIRM has been sporadic. After all, news outlets typically don't report on the work of scientists in the trenches.

In this blog, I have provided frequent coverage of HD science as well as related stem cell research. In my 15 years writing about HD science, I've learned that scientific progress *is* slow by nature. It's not just the CIRM projects that take a long time to produce results.

From my standpoint, stem cell science has produced a "growing array of possibilities" for treatments and the "potential for a new era in human health," as I noted after attending the 2013 World Stem Cell Summit (click here to read more).

Producing treatments is also extremely expensive. According to Jim Greenwood, president and CEO of the Biotechnology Industry Association, which organized the Bio Convention, developing a new drug in the U.S. costs an average of $1.2 billion. CIRM and/or its affiliated researchers will need to partner with the pharmaceutical industry to bring treatments to market.

In the HD community, we earnestly hope for stem cell treatments, but we're also aware that a "cocktail" of different approaches (like gene therapy) will likely be needed to deal with the complexities of the disease. We're rooting for *all* the researchers to find keys to treatments.

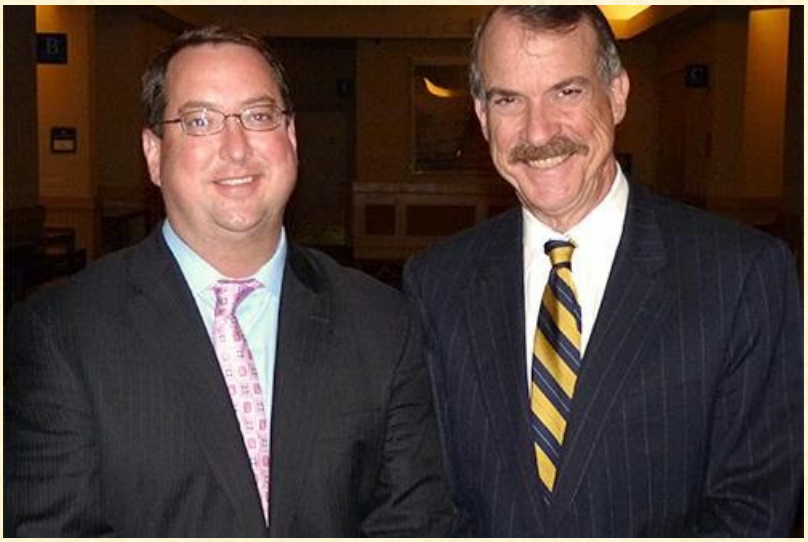
Crucial experience with clinical trials

With the need to show results, it's not surprising the CIRM board chose a new CEO from the business world.

As noted by David Jensen, author of the blog *California Stem Cell Report*, CIRM's previous two presidents, Zach Hall, Ph.D., and Alan Trounson, Ph.D., came from "largely academic and non-business backgrounds.... Decisions are likely to come faster under Mills."

In his introduction of Dr. Mills at the San Diego meeting, CIRM board chair Dr. Thomas said that the new CEO met the many qualifications sought by the organization, including familiarity with the process of running stem cell clinical trials and seeking approval of drugs from governmental agencies.

"Very few people can say they've had more experience in clinical trials in stem cells," Dr. Thomas said. "Very few people can say they've had more experience with the regulators, not just from the U.S., but from other countries as well."



Randy Mills (left) and CIRM board chair Jonathan Thomas (CIRM photo)

The board also sought someone familiar with CIRM. Dr. Mills has spent the last five years as a reviewer of proposals made to CIRM by stem cell researchers seeking funding. (Click here to read more.)

During the audience Q & A, one woman asked Dr. Mills what he would do to make the grant review process more "transparent."

Recognizing that the process wasn't "perfect," Dr. Mills nevertheless said he believed it was "pretty good" and already "remarkably transparent," with world experts involved in the reviews. He reminded the audience that no "divining rod" exists to pick perfect projects. He added that he will work for quicker approval of worthy applications.

Keeping CIRM running

Jeanne Loring, Ph.D., a leading expert on stem cells and Parkinson's disease at The Scripps Research Institute in San Diego, wanted to know how Dr. Mills would prioritize CIRM spending from now through 2017, when the last of the agency's grants will be made and the original CIRM allocation of $3 billion might run out.

The agency still has about $600 million in uncommitted funds. In all, $1.5 billion of its $3 billion budget has yet to be spent, as many budgeted projects remain in progress.

"Let's be careful on speculating on when CIRM is going to run out of money," Dr. Mills said in response to Dr. Loring's question. "That (2017)

would be the absolute earliest. This is an important thing for people to understand: in order for that date to be true, things have gone incredibly well. Everything we funded, 100 percent of it, has worked. If that '17 date happens, I'm a happy guy, because we are rattling off diseases left and right."

Dr. Mills explained that CIRM does "milestone-based funding."

"So we'll fund your project, but if you don't hit your milestone, if it's not working, we stop funding," he continued. "That seems like a pretty good idea. So the projections on these running out of money is assuming that everything is going along. Everything's going along, and we can't get California to say, 'Let's keep doing it'? In a more practical sense, we're not going to run out of money by then, and everything's not going to work perfect. My job is to run CIRM as efficiently as we possibly can to develop treatments."

According to spokesperson McCormack, the CIRM board can still redirect funding from the $1.5 billion as yet unspent. If a project comes in under budget, CIRM can also redirect savings to other projects, he added.

Some stem cell advocates such as Don Reed, who served on the executive board for the Prop 71 campaign, are already advocating a second round of CIRM funding to be requested from the state by way of another ballot proposition to be put before the voters. (You can watch Reed, HD advocate Judy Roberson, and children's neurological disorders advocate Alex Richmond speak about their experiences by clicking here.)

Dr. Thomas has also spoken publicly about seeking private sources of funding for CIRM. In this vein, Dr. Mills' experience in capital markets – one of the sought-for qualities in a CEO noted by Dr. Thomas – could prove helpful.

"California (undertook) a very important task in creating a funding stream for stem cell research," Clinton, referring to CIRM, said during her Q & A at the Bio Convention. "Other states have followed suit, when it looked as though the federal government would not be doing that. States have a role to play, but we need a national framework."

'Our urgency for cures'

Huntington's disease advocates participated in the "Meet the New CIRM President" events in San Diego as well as Los Angeles and San Francisco.

One of those participants, veteran advocate Frances Saldaña of Orange County, sees Dr. Mills' appointment as a positive step.

"I really liked Randy Mills," Saldaña, a mother of three children stricken with juvenile HD, told me in an e-mail about her encounter with Dr. Mills at the June 10 Los Angeles meeting. "I feel that he really understands our urgency to find cures."

Saldaña's daughter Margie Hayes – who became one of the very first HD patients to advocate for CIRM support for Huntington's stem cell research when she spoke at a December 2007 CIRM board meeting – succumbed to the disease on February 7. Hayes had just turned 44. She is survived by her husband Craig and two teenaged children.

Saldaña's husband also died of HD, which has afflicted several other members of her extended family. She was recently presented the 2014 Living Our Values Award by Michael Drake, the chancellor of the University of California, Irvine (UCI), for her work in HD community service. Saldaña is the founder of HD-CARE, an Orange County care organization affiliated with UCI's Institute for Memory Impairments and Neurological Disorders.

Saldaña said of Dr. Mills: “In the case of HD families, he completely understands that we're in a race against time, as our families are dying.”

As mother Frances Saldaña (left) looks on, Margie Hayes tells about her struggle against HD at the CIRM Spotlight on Huntington's Disease, Los Angeles, December 12, 2007 (photo by Gene Veritas).

Posted by Gene Veritas at 6:28 PM

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