

CIRM

CALIFORNIA'S STEM CELL AGENCY



OUR MISSION

Accelerating
stem cell treatments
to patients
with unmet
medical needs

“I am happy
to be part of it,
being a pioneer.
I get a warm
feeling knowing
I have been
a part of
this work.”

FRANK GONZALEZ,
CLINICAL TRIAL PARTICIPANT

Letter from the Chairman

JONATHAN THOMAS, J.D., PH.D.



2014 marked the 10th anniversary of the passage of Proposition 71, the ballot measure passed by 59 percent of the voters that formed CIRM and authorized \$3 billion to fund research in regenerative medicine.

By any measure, the past 10 years have seen California dramatically accelerate the pace of research in the field. Already we are funding 11 projects in human clinical trials with dozens of other exciting projects in the pipeline. CIRM monies, with significant philanthropic support, have funded research in 45 currently incurable diseases and triggered major initiatives in genomics, stem cell banks and one-stop "alpha clinics." We have helped build 12 state-of-art facilities, lured dozens of senior stem cell scientists to California and trained hundreds of future members of the biotech industry. California has become the global epicenter of stem cell research.

As to the future, with Alan Trounson's departure in June after 6 1/2 years at the helm, the Board welcomed Randy Mills as CIRM's next President and CEO. A 10 year stem cell veteran as CEO of Osiris Therapeutics and five year member of CIRM's peer review group, Randy has hit the ground running, taking stock of all that we have done and making it even better, all to further CIRM's mission of accelerating stem cell therapies to patients with unmet medical needs. We are delighted to have Randy aboard and look forward to even greater success in CIRM's second decade.

Letter from the President and C.E.O

C. RANDAL MILLS, PH.D.



It's been quite a year. As President and CEO of CIRM I strive to chart a clear course for the agency, one that builds on our rich past without taking our eye off the ultimate goal of transforming the practice of medicine through stem cells.

Our mission is simple; accelerate the development of stem cell treatments for patients with unmet medical needs.

With this in mind we set out to create CIRM 2.0. It's a radical overhaul of the way we do business that places emphasis on working with a sense of urgency. In the past it could take 22 months from application to funding; under CIRM 2.0 that number is less than four.

But CIRM 2.0 isn't just about speed. It's also about quality. We want to support great projects from start to finish, giving them the greatest chance to produce the life-saving, life-changing therapies we need. To meet this challenge, our team of highly trained and experienced professionals actively partners with academia, industry, and patients to fast-track the development of today's most promising stem cell technologies.

During my first year at CIRM, my greatest pleasure has been getting to know and work along side the extraordinary group of professionals who work here. They are smart, hard working, and committed to the mission and we couldn't have made as much progress in such a short space of time without their efforts.

At CIRM our mission is simple and clear, yet holds the power to transform the practice of medicine. We understand it will not be easy and that there can be no shortcuts along the way. But we also understand what is at stake and that failure is not an option.





A Stem Cell Pioneer

WHEN FRANK GONZALEZ WAS DIAGNOSED with colorectal cancer in November 2010 it was the start of a long fight against the disease.

Chemotherapy helped keep the cancer in check, but it wasn't a cure. So when Frank heard about a new experimental treatment, that seeks out and destroys cancer stem cells, he was intrigued.

Frank talked to UCLA's Dr. Zev Wainberg, who is running the clinical trial funded by CIRM: "I knew it was a study and everybody wasn't getting the same dosage but after having gone through all the other treatments this was easy."

Frank took a single pill every day, and says he experienced no side effects. After six months he had to drop out of the trial to receive radiation.

Frank's cancer is now in remission and he's been able to go back to work. He doesn't know if the pills helped but he's proud of being a stem cell pioneer and hopes the first-in-human therapy proves effective so that one day many others will be as lucky as he is.

"It is pretty amazing. I hope they close in on it. Figure this thing out, because there's a lot of need for it."

CIRM-Funded Clinical Trials

In 2004, 59 percent of California voters approved the creation of CIRM. Since that time, our research funding has helped move 11 projects into clinical trials; we also gave early support to eight other projects that resulted in clinical trials.

Before a new therapy is widely available in doctors' offices, it goes through carefully regulated testing in people. This happens in three stages:

Phase 1 trials: Determine safety of therapy in a small number of people

Phase 2 trials: Confirm safety of therapy in a slightly bigger group of people and begin to look for effectiveness

Phase 3 trials: Confirm effectiveness of therapy in large numbers of people

DISEASE FOCUS	TRIAL SPONSOR	TRIAL STATUS
BLINDNESS / MACULAR DEGENERATION	USC	PHASE 1
BLINDNESS / RETINITIS PIGMENTOSA	UC IRVINE	PHASE 1/2
CANCER / LEUKEMIA	UC SAN DIEGO	PHASE 1
CANCER / SOLID TUMOR	STANFORD	PHASE 1
CANCER / SOLID TUMOR	UCLA	PHASE 1
DIABETES	VIACYTE	PHASE 1
HEART DISEASE	CAPRICOR	PHASE 2
HIV/AIDS	CALIMMUNE	PHASE 1/2
HIV/AIDS	CITY OF HOPE/SANGAMO BIOSCIENCES	PHASE 1
SICKLE CELL DISEASE	UCLA	PHASE 1
SPINAL CORD INJURY	ASTERIAS BIOTHERAPEUTICS	PHASE 1/2

RESEARCH FUNDING STATS

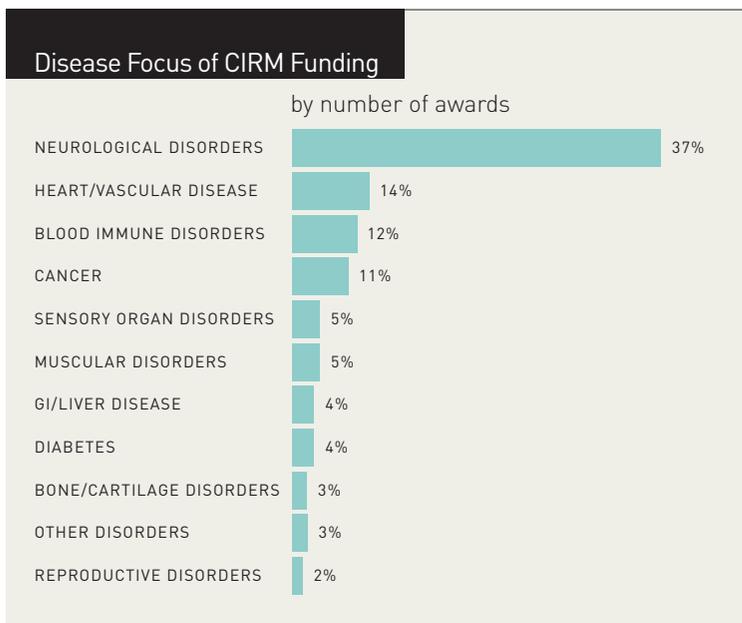


Funding to accelerate stem cell treatments for unmet medical needs

Remaining capital to fund awards valued up to \$20M

In one decade CIRM had 11 therapies approved for clinical trials

Active stem cell programs in CIRM's portfolio



- Research Funding in 2014: **\$143 million**
- Awards granted in 2014: **37**
- Total funding awarded: **\$1.93 billion***
- Total funding disbursed: **\$1.52 billion***
- Total awards granted: **652***

**As of April 2015*

For more details on CIRM's funding commitments visit:
<https://www.cirm.ca.gov/our-progress/cirm-funding-commitments>

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