THE STATE STEM CELL AGENCY

C I R M

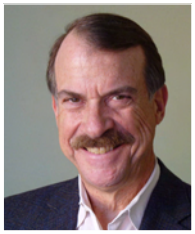
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE ANNUAL REPORT 2011

Turning Stem Cells into Cures

LETTER FROM THE CHAIRMAN

Jonathan Thomas, J.D., Ph.D.

THE NEXT PHASE: TRANSLATING INVESTMENT INTO THERAPIES



It was with a great sense of pride, privilege and anticipation that I took over for Bob Klein as the second Chairman of CIRM's governing board this past June. His were difficult shoes to fill. In his time as chair Bob had overseen initiatives that made California the "Stem Cell State" and the epicenter for stem cell research in the world.

When I became chair, CIRM's programs had awarded $1.3 billion to 57 different institutions, with a significant portion of it in 43 major translational awards targeting 26 incurable diseases. The agency was also well on the way to meeting projections of creating 25,000 job years and $200 million in tax revenue by 2014. All this while capping administrative costs at six percent, unheard of for a state agency.

Now, CIRM is poised for a new phase in its development: translating the investment of its initial years into therapies. That process takes time and not every effort will lead to a new cure. But we firmly believe that a number of our projects will succeed and that every single breakthrough will be a game changer.

Thank you once again California for this unparalleled opportunity to change the world.

"CIRM is poised for a new phase in its development: translating the investment of its initial years into therapies."

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

LETTER FROM THE PRESIDENT

Alan Trounson, Ph.D.

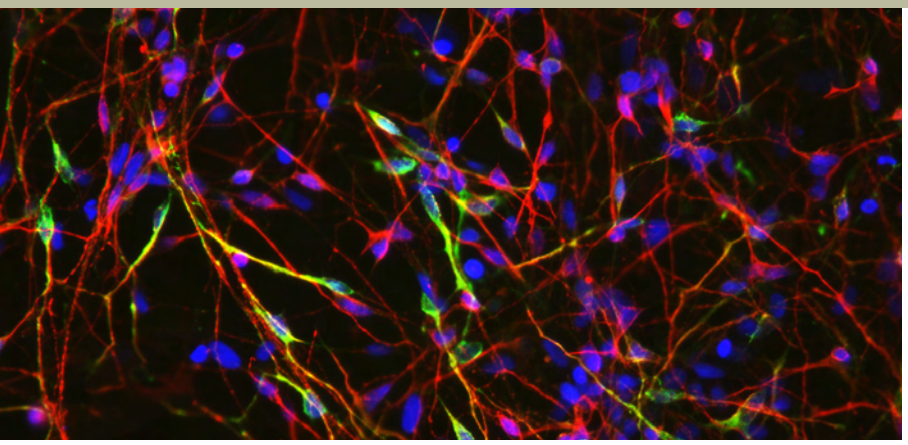
BUILDING THE STEM CELL RESEARCH FREEWAY: DESTINATION PATIENTS

CIRM has created a stem cell research freeway that will deliver therapies to patients. In 2011 CIRM began building the interchanges that allow stem cell researchers to access complementary expertise that can accelerate the field. We also started creating the off ramps that will take research projects to their final destination—the patients.

In addition to critical scientific advances, which you can read about in my full letter online, we launched programs to create cell banking and genetics resources. These will allow our grantees to focus on finding cures, rather than replicating existing expertise.

With the knowledge that biotechnology partnerships will be critical to the success of our grantees, we launched a trio of programs called the Opportunity Funds that seek to ensure projects that are most likely to make it into clinical trials have sufficient capacity, expertise and financial support for this demanding work.

These programs will help us deliver on the extraordinary revolution in science and medicine envisioned by those who created and voted for proposition 71.



Xianmin Zeng, Buck Institute for Age Research

NEWS AT CIRM

2011 IN REVIEW: CREATING JOBS, SPEEDING RESEARCH AND MAKING PROGRESS TOWARD THERAPIES

Read the full stories, watch videos and get links to more information about our progress toward therapies in the annual report online:
www.cirm.ca.gov/2011AnnualReport

Eliminating HIV
Two years ago the first person was cured of HIV, but the bone marrow transplant that cured him can't be scaled up to help all infected people. Now, two CIRM-funded teams are mimicking that treatment by manipulating a person's own blood-forming stem cells to make them resistant to HIV. Several advances this year suggest those researchers might be on to something.

"These programs will help us deliver on the extraordinary revolution in science and medicine envisioned by those who created and voted for proposition 71."
ALAN TROUNSON, PH.D., PRESIDENT

Smoothing the path
Developing a new therapy takes more than just clever science. Navigating the many hurdles leading up to and throughout a clinical trial requires an expert touch. CIRM is actively linking our grantees with that experience to help make sure their good ideas reach California patients.

Diabetes progress
Diabetes seems like a disease custom made for a stem cell therapy: Replace the insulin-producing cells that are destroyed by the body's immune system with ones derived from embryonic stem cells. But how do you protect those replacements from attracting the immune system? A CIRM-funded team lead by San Diego company ViaCyte thinks they have an answer—it's so promising that JDRF recently co-funded the project.

Banking on stem cells
Want to study Huntington's disease neurons? Test drugs for Parkinson's disease or heart disease? Unravel the origins of ALS or Alzheimer's disease? First you will need stem cells created from people with those diseases. Our banking partnership with the NIH and a California-based program will generate, store and distribute such cells to help scientists understand and eventually treat genetic diseases.

Go west!
In early 2010 Robert Wechsler-Reya was running a research lab at Duke University investigating the stem cell origins of a form of childhood brain tumor. Today, he is at the Sanford-Burnham Medical Research Institute in La Jolla, where his work has already led to a discovery that could point to new therapies. He's one of many leading scientists and companies that are relocating to California—and bringing jobs in their wake—lured by California's commitment to stem cell research.

Spotlight on Diseases

At governing board meetings, a Spotlight on Disease presentation features patients, clinicians and researchers speaking about the hope of stem cell research.

Read their stories online: **www.cirm.ca.gov/2011AnnualReport**

NEUROMYELITIS OPTICA

"Stem cells would be an important piece of the potential therapeutics for NMO. The work that CIRM is doing is really important."

VICTORIA JACKSON, MOTHER OF A GIRL WITH NMO

SICKLE CELL DISEASE

"This research is something that can really make difference in the lives of kids and families and I certainly cannot thank you enough for making it happen."

NANCY RENE, GRANDMOTHER OF A CHILD WITH SICKLE CELL DISEASE

AUTISM

"I think [stem cell research] is going to revolutionize a lot. It will revolutionize the way we look at disease."

LAUREEN FORMAN, MOTHER OF A CHILD WITH AUTISM

HEART DISEASE

"It's given me a new life. The process, from the patient's point of view, is an easy, painless miracle, and I'm very thankful."

FRED LESIKAR, HEART ATTACK SURVIVOR

"The patient advocate board members have an incredible ability to think about who will benefit from our funding decisions because it's personal. They always have that focus—will this help get products to me or not?"

DUANE ROTH, CIRM CO-VICE CHAIR

TRAINING THE NEXT GENERATION

CALIFORNIA HIGH SCHOOL, UNDERGRADUATE AND GRADUATE STUDENTS WILL BECOME TOMORROW'S STEM CELL SCIENTISTS.

CIRM has programs to bring high school, undergraduate and master's students—many from economically challenged backgrounds—into stem cell science careers.

Our programs also train graduate and post-graduate students to run their own stem cell labs. These students will be tomorrow's stem cell scientists, continuing California's leadership in developing therapies for incurable disease and injury.

"The Bridges to Stem Cell Research and Creativity Award programs train the next generation of California stem cell scientists by providing our state's young people opportunities to reach their highest potential through life changing medical research."

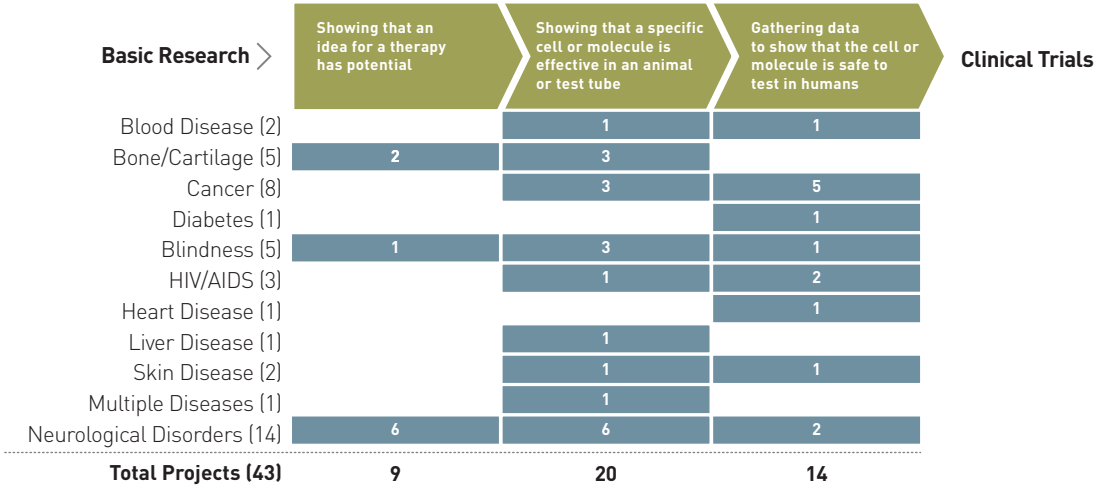
- CALIFORNIA SENATOR ART TORRES (RETIRED)
STATUTORY VICE-CHAIR, CIRM

PROGRESS TOWARD THERAPIES

CIRM-FUNDED PROJECTS NEARING PATIENTS

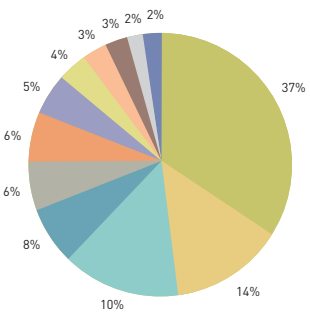
CIRM funds all phases of research from basic science that produce the breakthrough ideas all the way through filing paperwork to begin clinical trials. So far, 43 of our projects in 26 disease areas are in various stages of working toward clinical trials. Here is a summary of how many projects are in different stages of moving down the path to the clinic.

Therapy Development Pipeline



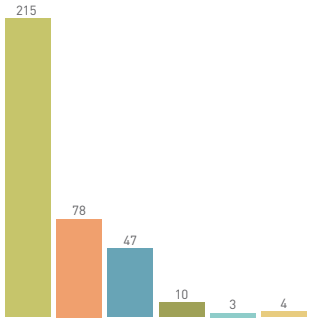
FINANCIAL

SUPPORTING ALL STEM CELL TYPES IN THE SEARCH FOR CURES



Disease Category
By award number

- Neurological disorders
- Cancer
- Heart disease
- Blood/immune disorders
- Other disorders
- Sensory organs
- Multiple disorders
- Muscular disorders
- GI/liver disease
- Bone/cartilage disorders
- Diabetes
- Reproductive disorders



Stem Cell Type Funded
By award number

- Embryonic
- Reprogramed iPS cell
- Adult
- Cancer
- SCNT
- Other

Accomplishments

Putting California funds to work:

- Funds leveraged from private donors/ institutions: $884M
- Funds leveraged from collaborative partners: $60M
- Job years forecast by 2014: 25,000
- Tax revenues forecast by 2014: $200M

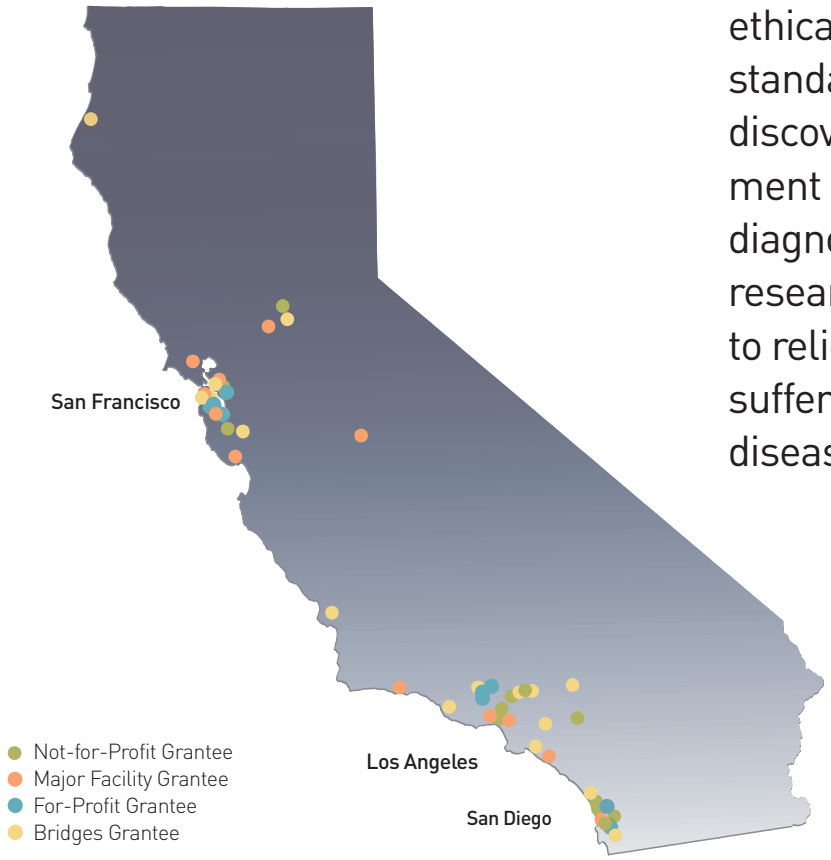
For more information on these successes and for a full report on the CIRM financials see the annual report online: **www.cirm.ca.gov/ 2011AnnualReport**

the mission of CIRM

To support and advance stem cell research and regenerative medicine under the highest ethical and medical standards for the discovery and development of cures, therapies, diagnostics and research technologies to relieve human suffering from chronic disease and injury.

STEM CELL GRANT RECIPIENTS

CIRM GRANTS THROUGHOUT CALIFORNIA SUPPORT STEM CELL RESEARCH, TRAINING AND FACILITIES ALL WITH THE GOAL OF DEVELOPING NEW DISEASE THERAPIES.



Read about our progress toward achieving that mission:
www.cirm.ca.gov/2011AnnualReport

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