

THE STATE STEM CELL AGENCY

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE ANNUAL REPORT 2013

CIRM

Turning
Stem Cells
into
Cures

JOSHUA GOULART
HEART DISEASE



Letter from the Chairman

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

ACCELERATING OUR PROGRESS



This past year the agency refined both its direction and grant-making process. With a goal of advancing the most promising projects as quickly as possible the Board approved an “accelerated pathway” program. The goal is to get as many projects as possible to

Phase 2 clinical trials and show these therapies work. After that big pharma, biotech and venture capital investors can take things into Phase 3 trials and hopefully, onto FDA approval and use in patients.

On the process front, the year began with a report from the Institutes of Medicine that called the agency “a bold social innovation” adding “the work of CIRM-sponsored researchers continues to enrich regenerative medicine everywhere.” But the report also highlighted a number of issues including perceived Board conflicts of interest and needed changes to CIRM’s appeals procedures. To address all those concerns the Board adopted a comprehensive set of changes at its very next meeting, which dramatically refined the way CIRM does business.

In August we mourned the death of longtime Board member and a great friend to the agency, Duane Roth. His wisdom and expertise were a vital part of everything we did. We miss him greatly.

With many of our projects showing great progress we expect this will be yet another banner year in the march towards therapies and cures.

“California has led the way
with stem cell research”

GOVERNOR JERRY BROWN, 2014 STATE OF THE STATE SPEECH

“Thanks for the privilege and the
opportunity to make a difference.”

ALAN TROUNSON, PH.D., PRESIDENT

Letter from the President

Alan Trounson, Ph.D.

LONG MARCH TO THE CLINIC REACHING THE DESTINATION



My final full year as president of CIRM turned out to be an incredibly fulfilling one with the agency awarding \$63 million to six projects to begin clinical trials, two previously funded projects launching clinical trials and a raft of projects gearing up for trials.

I feel like the undertaking I began when Governor Arnold Schwarzenegger and Robert Klein gave me the privilege of leading this institute was to deliver the benefits of stem cell discoveries to patients in need. I was allowed to approach this goal without undue political interference, and now our work is beginning to reach patients. That was the dream that launched the institute a decade ago. I feel satisfied that the primary job I agreed to do is done.

Last spring we provided over \$6 million to begin a clinical trial for beta-thalassemia. Then we closed the year by awarding \$57 million to five Disease Teams to launch clinical trials for solid tumors, blindness, sickle cell anemia, and two different approaches to leukemia. Two prior projects began clinical trials this year, one for patients with serious damage after a heart attack and one for curing patients with HIV.

We have just created a powerful new center of excellence that will enable the best genomics researchers to work with the best stem cell biologists. I predict this will result in some of the major discoveries of the next few decades.

We also launched a valuable tool for researchers worldwide last year: the creation of an 9,000-line bank of cells destined to change our understanding of 11 diseases.

I will be stepping down as president of CIRM sometime in 2014, but I can honestly say that working to meet the goals of the voters who created CIRM has been one of the most fulfilling chapters in my career. Thanks for the privilege and the opportunity to make a difference.



Stories of Hope

IN HER LATE TWENTIES NAN LUKE'S ENTIRE LEFT SIDE WENT NUMB WITHOUT WARNING.

The diagnosis was multiple sclerosis (MS) and thirty years later the chronic pain from invisible pins and needles hasn't gone away.

Now CIRM funded researchers are making progress to mature stem cells into a type of cell that could be transplanted into a person with multiple sclerosis to repair damage caused by the disease. See more stories of hope online: www.cirm.ca.gov/hope

"This basic science translates into hope for us and treatment for MS and other diseases, and I myself am grateful. Researchers rock!"

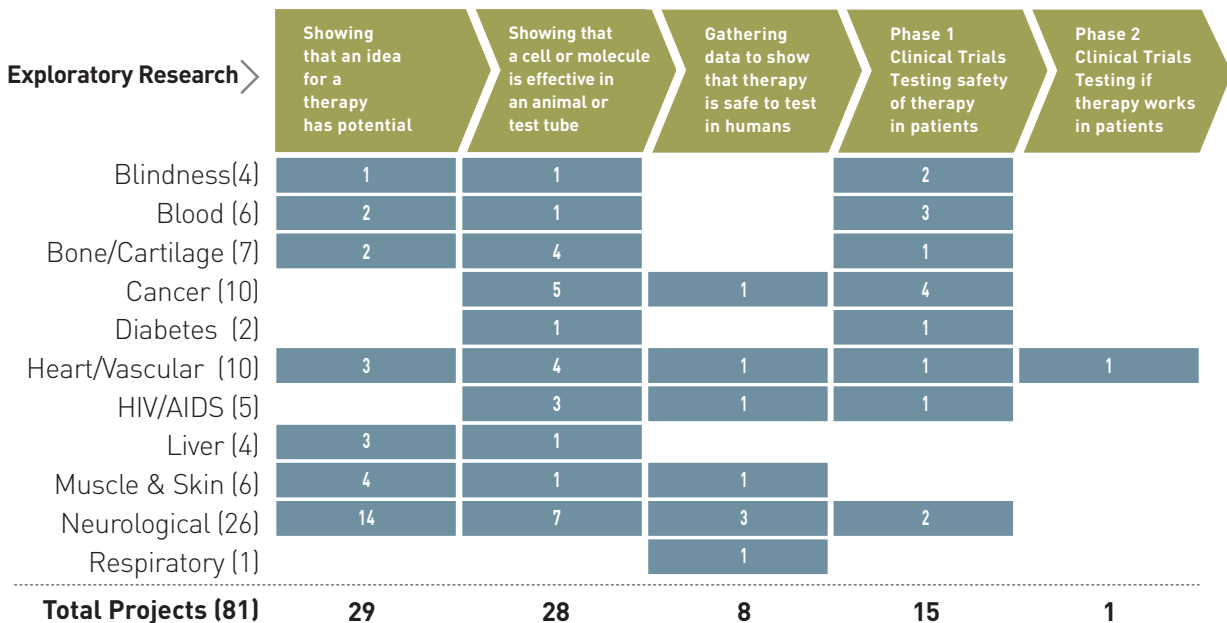
NAN LUKE, MULTIPLE SCLEROSIS PATIENT ADVOCATE

Progress Toward Therapies

CIRM-FUNDED PROJECTS NEARING PATIENTS

CIRM funds all phases of research from basic science that produces breakthrough ideas all the way through to testing whether potential therapies are safe and effective in patients. So far, 81 of our projects in 39 disease areas are in various stages of working toward clinical trials.

Therapy Development Pipeline



Our Accomplishments

CLINICAL TRIALS:

CIRM funding has resulted in **10** therapeutic approaches being tested in clinical trials.

TRAINING YOUNG PEOPLE:

We've trained more than **1,500** high school, college, graduate, and postdoctoral level students.

TAX REVENUES FOR CALIFORNIA:

Forecasts estimate we will have generated **\$286M** in new tax revenue by 2014.



"Thank you for ALL of your support over the past several years. We wouldn't be where we are today without you."

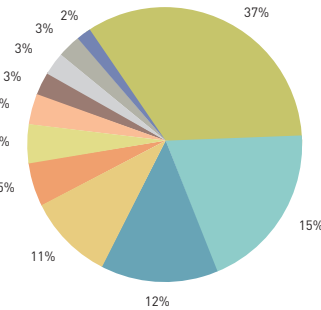
EDWARD LANPHIER, SANGAMO CEO AND PRESIDENT - speaking about Sangamo's agreement with a global pharmaceutical company to bring a stem cell treatment for sickle cell anemia to clinical trials.

CIRM Research Funding

- Funding awarded in 2013: \$163M
- Total funding awarded: \$1,827M
- Total funding dispersed as of Feb 2014: \$1,266M
- Awards granted in 2013: 36
- Total awards granted: 607

Learn more about where our funding goes:

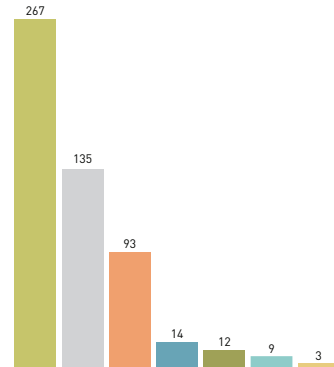
www.cirm.ca.gov/grants



Disease Category

By award number

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



Stem Cell Type Funded

By award number

- Embryonic
- Reprogrammed iPS cell
- Adult
- Cancer
- Other
- Direct reprogramming
- SCNT