2015 Annual Report

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CIRM 2015 ANNUAL REPORT

OUR MISSION | To accelerate stem cell treatments to patients with unmet medical needs.

Letter from the Chairman

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

What a difference a decade makes. The CIRM governing Board approved the agency's first grants in September of 2005. Those were 16 modest awards totaling $12.5 million to train a new generation of stem cell researchers. But even those awards were not funded until the following April when the Board arranged a novel funding mechanism through donors; a move necessitated by an ongoing court case that prevented issuance of the bonds intended to fund our work. Fast forward to 2015 and CIRM awarded 47 grants totaling $135 million in commitments to research at all levels.

Also in 2005, the Board began soliciting bids from California cities to host our headquarters, a contest San Francisco won. After 10 years of gracious hosting by San Francisco, 2015 saw us move across the Bay to new offices in Oakland at considerable cost savings for the voters of California. A truly formative year for CIRM, in 2005 the agency began a public hearing process on its direction that ultimately resulted in adoption of our first strategic plan in December the following year. After two revisions of that plan, in 2015, our new President Randy Mills embarked on a listening tour of all our constituents that produced a radically new strategic plan adopted by the Board unanimously in December 2015.

During that first decade of grant making, we saw the entire field accelerate and by the end of 2015 CIRM had funded 15 clinical trials and most observers of the field, including a report from the National Academies of Science credited CIRM with being a key player advancing the pace of stem cell research. However, with our new strategic plan we are ratcheting up that effort. We have a goal of funding 50 more clinical trials in just five years. But with Randy and the entire team of dedicated professionals at CIRM, as well as a fully committed Board, I have no doubt that we will make the best effort possible to hit this goal. The patients with unmet medical needs highlighted in our mission statement deserve no less.

Letter from the President and CEO

C. RANDAL MILLS, PH.D.

Better. That is what we are fighting for. Better infrastructure to translate early stage ideas into groundbreaking clinical trials. Better regulatory practices to advance promising stem cell treatments more efficiently. Better treatments for patients in need.

But better is also a standard we need to continuously hold ourselves to at CIRM. That is why in 2015, we undertook a complete review of the organization; where it came from; where it was; and most importantly, where we wanted it to be. Every idea was considered. Nothing was out of bounds.

The result is our new Strategic Plan, unanimously adopted by our governing Board this past December. It charts a bold new course for the stem cell agency, with clear goals for us to achieve over the next five years.

It's a pretty ambitious plan; it includes funding 50 new clinical trials, discovering 50 new therapeutic candidates, and cutting in half the time it takes to move a promising idea into the clinic where it can be tested in people. We know meeting those goals won't be easy - nothing truly worthwhile ever is - but we are confident we can succeed. We have an amazing team here at CIRM, one that is committed to our mission of developing treatments for patients with unmet medical needs.

In the coming months and years, we will be reporting our progress on this plan, back to you, our stakeholders. This part of the process is imperative, because without laser-like focus on results we have little chance of success.

We are in a race against time and we know it. Too many patients are suffering. Too many people are running out of options. We are committed to doing all we can to change that. We are committed to better.

CIRM-FUNDED CLINICAL TRIALS

In 2004, 59 percent of California voters approved the creation of CIRM. Since that time, our funding has helped move **18 projects into clinical trials**; we also gave early support to 10 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 generally 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

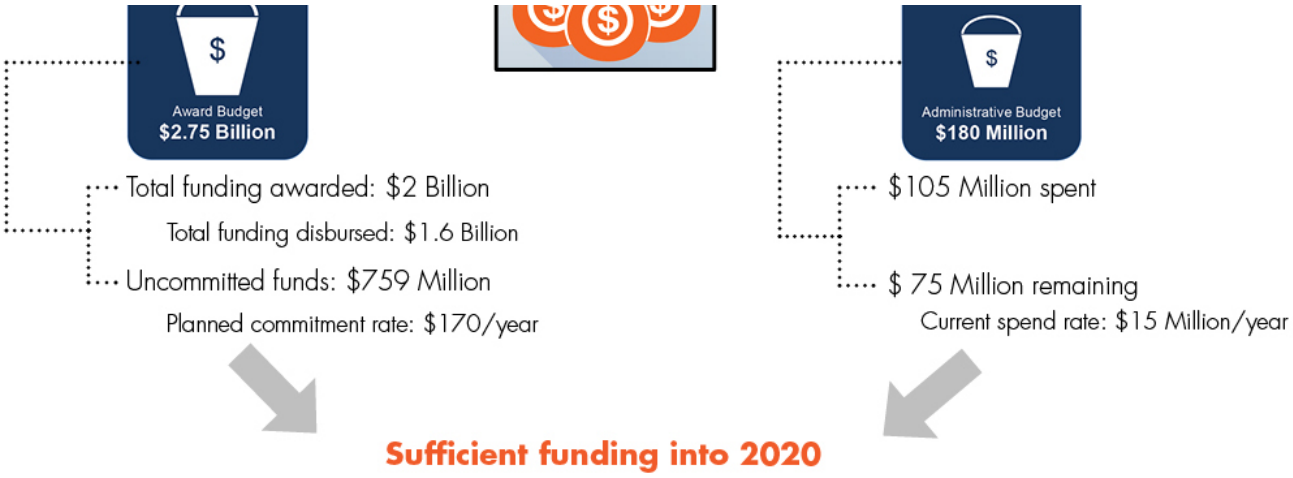
DISEASE FOCUS	TRIAL SPONSOR	TRIAL STATUS
BLINDNESS / MACULAR DEGENERATION	USC	PHASE 1
BLINDNESS / RETINITIS PIGMENTOSA	jCYTE	PHASE 1/2
CANCER / BRAIN	IMMUNOCELLULAR	PHASE 3
CANCER / LEUKEMIA	UC SAN DIEGO	PHASE 1
CANCER / SOLID TUMOR	STANFORD	PHASE 1
CANCER / SOLID TUMOR	UCLA	PHASE 1
DIABETES	VIACYTE	PHASE 1
HEART DISEASE	CAPRICOR	PHASE 2
HEART DISEASE RELATED TO DUCHENNE MUSCULAR DYSTROPHY	CAPRICOR	PHASE 1/2
HIV/AIDS	CALIMMUNE	PHASE 1/2
HIV/AIDS	CITY OF HOPE / SANGAMO BIOSCIENCES	PHASE 1
HIV/AIDS	UC DAVIS	PHASE 1/2
HUNTINGTON'S DISEASE	UC DAVIS	OBSERVATIONAL
IMMUNE DISORDER / CHRONIC GRANULOMATOUS DISEASE	UCLA	PHASE 1
SICKLE CELL DISEASE	UCLA	PHASE 1
SPINAL CORD INJURY	ASTERIAS BIOTHERAPEUTICS	PHASE 1/2
CANCER / SKIN	CALADRIUS	CLOSED
SPINAL CORD INJURY	GERON	CLOSED

For more details, visit our CIRM-Funded Clinical Trials page

CIRM RESEARCH FUNDING STATS (as of Dec 2015)

Financial Overview

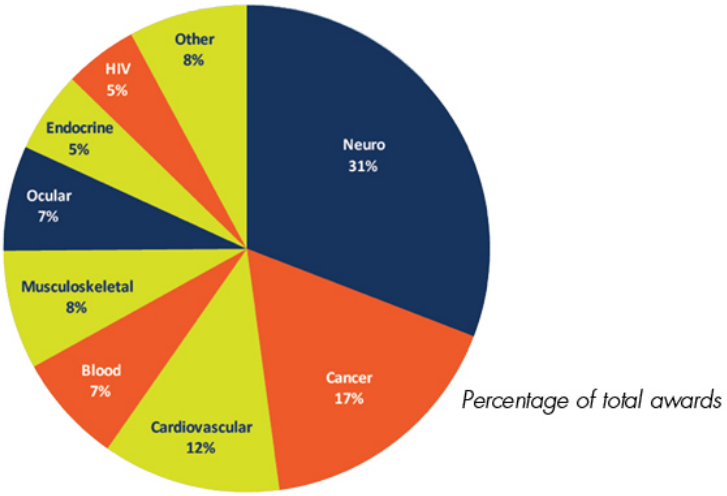
Proposition 71 which created CIRM in 2004 authorized $3 Billion in funding – through the sale of state bonds – for stem cell research in California. Here is an overview of those funds as of December 2015:



Snapshot of 2015 Funding

Disease Focus of CIRM Funding

As of the December 2015, CIRM has invested $1.3 billion in disease specific research across a wide range of organ and tissue systems



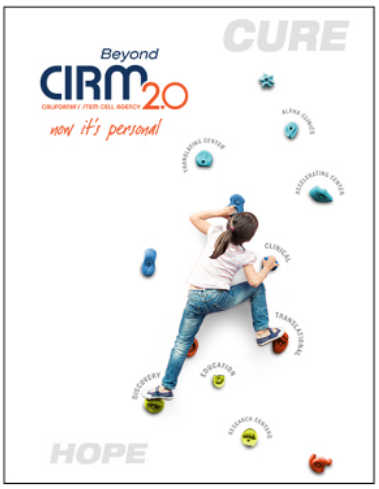
For more details about CIRM financial data visit:

- **CIRM's Funding Commitments Page**

- **CIRM's Annual Financial Statements**

2016 and Beyond: CIRM'S Five Year Strategic Plan

We didn't want something 'good enough', we wanted something 'great'. This plan is extremely ambitious, but also realistic in the goals it sets out and the way those goals can be met." – C. Randal Mills, Ph.D.



Download Strategic Plan [pdf]

In 2015, under the leadership of President and CEO C. Randal Mills, Ph.D. the Institute launched CIRM 2.0 – a radical overhaul of the way the agency operates. Specifically, CIRM 2.0 introduces faster, more efficient systems and programs that place added emphasis on speed, partnerships and patients.

With the launch of CIRM 2.0 underway, the CIRM governing Board approved a new Strategic Plan in December 2015 to create a clear vision for the next five years of the Institute. The Plan include six major goals, affectionately called The Big 6, that maximizes the Institute's chances of pushing as many new treatments to patients with unmet medical needs as possible.

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