CURED

Evangelina Padilla Vaccaro

Previously diagnosed with severe combined immunodeficiency disease

Challenges remain,
but new cures
are emerging.

All In. All Out.

CIRM
CALIFORNIA'S STEM CELL AGENCY

2016 Annual Report

MISSION

Accelerate stem cell treatments to patients with unmet medical needs

About CIRM

The California Institute for Regenerative Medicine (CIRM) arose from the vision of California's people: that stem cell treatments could provide new options and greater hope to patients with serious unmet medical needs.

Patient advocates joined forces with doctors and scientists to create a vehicle to fund stem cell research in California. On November 2, 2004, 59 percent of the voters in this state approved Proposition 71, which amended the California Constitution to establish the right to conduct stem cell research in California, authorized $3 billion to fund stem cell research, and created CIRM.

In authorizing these funds, Californians expect to speed the development and delivery of stem cell treatments and cures to patients, including making it a priority to fund ethical research that was not receiving timely or sufficient federal funding.

CIRM's governing Board, the Independent Citizens' Oversight Committee (ICOC), continually works to further CIRM's mission while providing financial responsibility to the people of California.

The ICOC—which includes representatives from patient advocacy groups, medical research, and industry—is charged with adopting scientific, medical, ethical, and intellectual property policies; making final funding decisions on grant and loan awards; and providing oversight of CIRM.

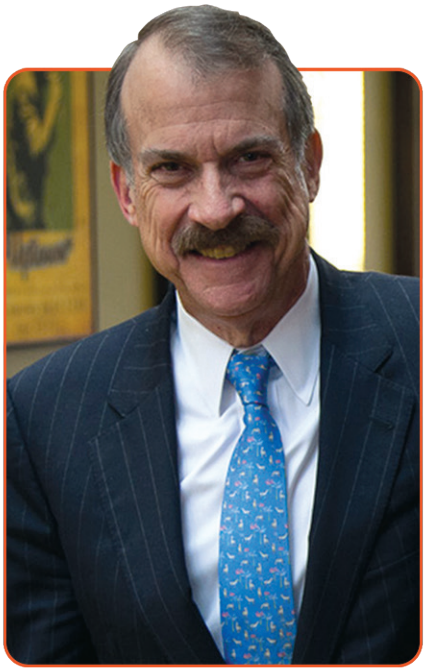
CIRM is focused on achieving its mission through the timely and efficient funding of high-quality stem cell projects and accelerating delivery of resulting treatments and cures to patients in need.

CIRM is singularly committed to this goal. Because every moment counts.

CIRM at a Glance

- Formed in 2004 through passage of Proposition 71
- Amended the California Constitution to create a right to conduct stem cell research
- Created a $3 billion fund for stem cell research
- Engaged patients, advocates, physicians, scientists, and industry leaders in the decision-making process
- Enabled 27 stem cell clinical trials
- Constructed 12 world-class research facilities and three stem cell Alpha Clinics across the state
- Partnered to create the first stem cell-specific Translating and Accelerating Centers
- Produced more than 180 inventions to date
- Currently manages over 250 active projects

Looking Back



Friends,

Looking back at what has been accomplished over the last 12 years, the people of California have a right to feel proud. In 2004 California acted boldly by overwhelmingly passing Proposition 71, the California Stem Cell Research and Cures Act, creating and funding the California Institute for Regenerative Medicine (CIRM).

Today we have a tremendous amount to show for it, including world-class research facilities, a unique network of high-quality Alpha Clinics to conduct stem cell clinical trials, and a new generation of stem cell researchers. In carrying out the mandate of Proposition 71, we have solidly established the State of California as the global leader in regenerative medicine.

However, there can be no greater return on our investment than the restoration of health that is now occurring because of CIRM. Evangelina (featured on the cover), stricken with a previously incurable immune system disorder, is now cured. That's right, cured. And we are working to quicken the pace at which this treatment is available to others. This is the promise of stem cell therapy. This is the promise of CIRM.

We are witnessing the dawn of a new age in medicine—one that offers real hope to many who previously had little. This revolution is happening thanks to the foresight of the people of California. And our great team at CIRM is ALL IN!

Sincerely,

Jonathan Thomas

Jonathan Thomas, Ph.D., J.D.
Chairman, Independent Citizens' Oversight Committee

We embrace an **ALL IN, ALL OUT** culture and commitment to achieving our goals, as if someone's life depends on it—because it does.

Looking Forward

Partners,

Looking forward, we can now see cures and treatments on the horizon for some of the most vexing challenges in medicine.

CIRM is aligned around a single, clear mission: to accelerate stem cell treatments to patients with unmet medical needs. When so much force is applied, all in the same direction, tremendous progress is possible. That is why we developed and implemented our current strategic plan—Beyond CIRM 2.0—and the results could not be more encouraging:

- Within the last two years we have added 17 high-quality clinical trials to our portfolio;
- We have increased the number of projects we fund each year by 33 percent; and
- We have reduced the time it takes to approve an award by 82 percent.

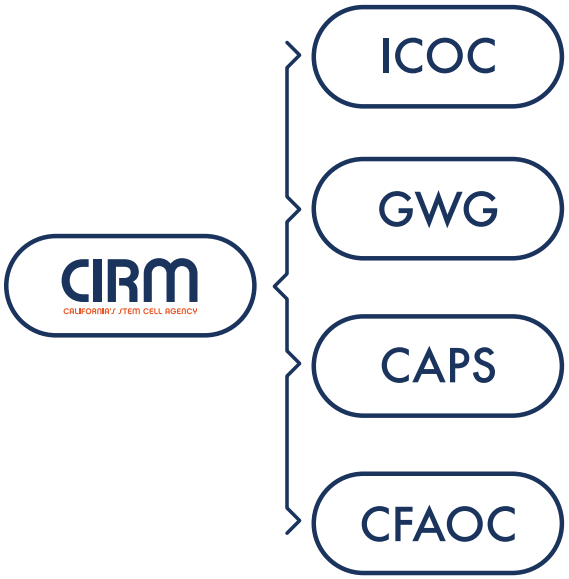
These tremendous results were delivered by our amazing and dedicated team, but are only possible because of patients who partake in the testing of potential new treatments. They all enter clinical trials knowing there are real risks, but selflessly participate so that others may benefit. They are true heroes, and this report is dedicated to their service.

At CIRM, we act as if lives depend on it, because they do. We will never stop improving. We will never stop looking for ways to do more, faster, with greater success. We can see the future of medicine. And at CIRM, we are going ALL OUT to make it a reality as quickly as possible.

Sincerely,

C. Randal Mills, Ph.D.
President and Chief Executive Officer

CIRM Reporting Structure



Independent Citizens' Oversight Committee—the 29-member governing Board composed of leaders in California from the patient advocacy, biotechnology, and medical research sectors.

Grants Working Group—a panel of experts, all from outside California, who review grant applications for scientific merit and recommend to the ICOC those they believe should be funded.

Clinical Advisory Panels—panels created to provide insight, guidance, and support to clinical stage projects. Each consists of CIRM Science Officers, external experts in the field, and at least one patient representative to provide our clinical programs the greatest chance of success.

Citizens Financial Accountability Oversight Committee—the committee, led by the State Controller, to review the financial practices of CIRM.

Our Investment Programs

INFRASTRUCTURE

The CIRM Infrastructure Program builds real and virtual centers that provide the resources, expertise, and information necessary to more efficiently advance CIRM's programs and projects.

EDUCATION

CIRM's Education Programs support the development of a workforce qualified to drive achievement of the CIRM mission, now and in the future.

DISCOVERY

The Discovery Program supports the exploration of new, potentially ground-breaking stem cell treatments and technologies from inception to translation.

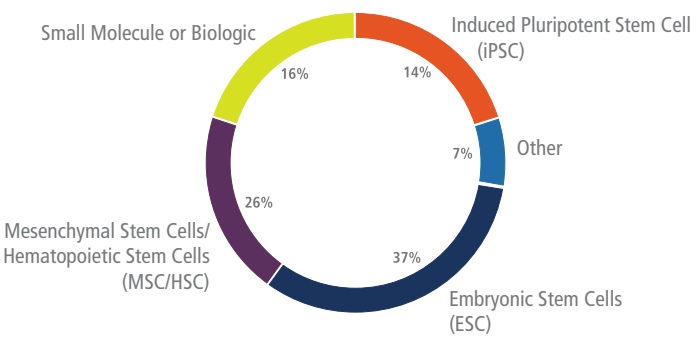
TRANSLATION

The Translation Program supports the acceleration of early development activities necessary to prepare stem cell treatments, devices, or tools for clinical study.

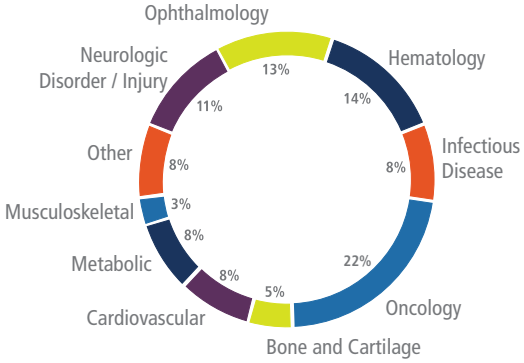
CLINICAL

The Clinical Program supports the acceleration of high-quality clinical trials of stem cell treatments to address unmet medical needs.

Awards by Technology Type



Awards by Disease Type



All In *All Out*

Students trained in CIRM's educational programs **2,432**

Stem cell lines in CIRM's new iPSC bank **1,211**

Projects funded since CIRM's inception **754**

Stem cell clinical trials funded by CIRM **27**

Major stem cell research centers constructed in California **12**

CIRM-created stem cell Alpha Clinics **3**

1 Reason *Why*

to speed stem cell treatments to those who need it most

Brenden Whittaker

Age 1: Diagnosed with severe chronic granulomatous disease, a rare disease that causes the immune system to malfunction, leading to recurring bacterial and fungal infections that can be life-threatening.

Age 23: CURED

"It's weird being healthy. Before this year, I was sick pretty consistently, going to doctor's appointments two or three times a week and being in the hospital.

Today, I find enjoyment in the small things, things I couldn't do before, but now I can and not have to worry about them. Cutting the grass is something I couldn't do before that I've taken up now. Most people look at me as if I'm crazy when I say it, but I love cutting grass. I wasn't able to do it for 22 years of my life.

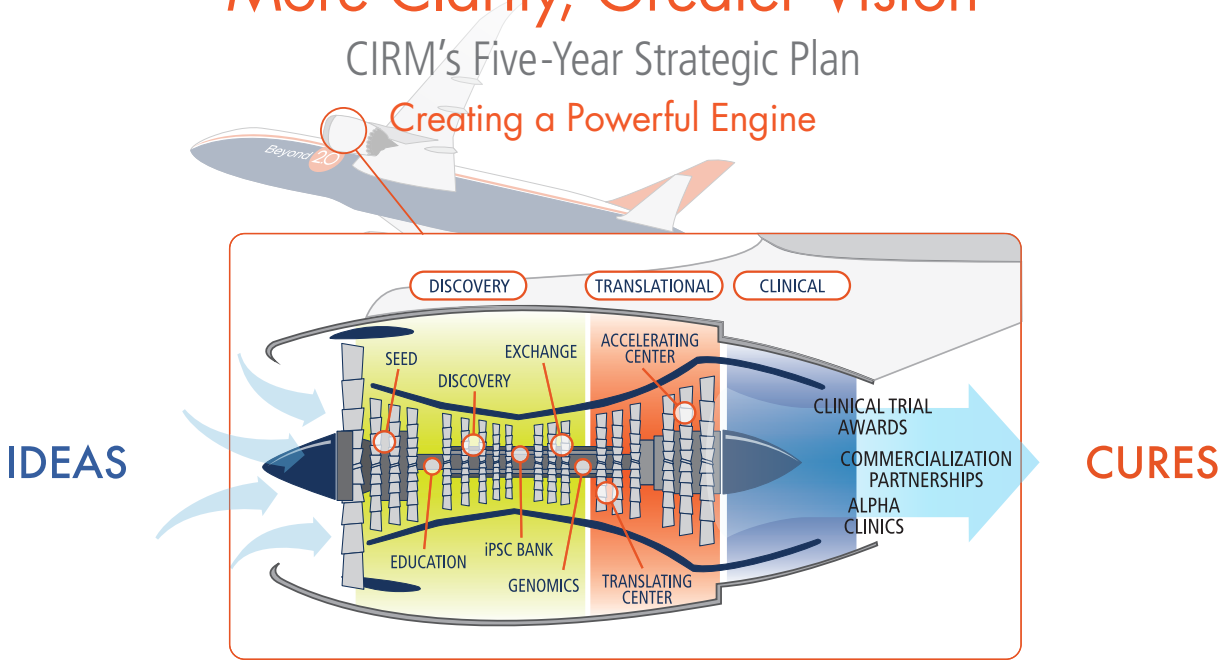
I'm getting ready to go back to school full time. I want to get into pre-med, go to medical school, and become a doctor. All the experience I've had has just made me more interested in being a doctor. I just want to be in a position where I can help people going through similar things to what I experienced.

For people going through anything like this, don't give up. There are new advances being made every day. Keep fighting. Keep moving forward. Someday it will all work out."

More Clarity, Greater Vision

CIRM's Five-Year Strategic Plan

Creating a Powerful Engine



VOLUME | SPEED | QUALITY

CIRM has implemented a strategic plan to maximize the agency's impact over the five-year period from 2016 to 2020. We are creating an engine that increases the volume, speed, and quality of promising new stem cell ideas and turns them into reality. The plan is bold, but with a great team all pulling in the same direction, tremendous accomplishments are possible.

The purpose of the plan is to create and maintain alignment among CIRM's stakeholders by connecting the dots from the "big picture" mission to specific actions, goals, and priorities.

To accomplish this we made four key changes to the system in 2016:

- Standardized our recurrent program offerings. Discovery, translation, and clinical programs are now offered on a predictable and repeatable schedule, available when the science is ready.
- Increased speed and cycle frequency. We now review applications faster and more frequently than ever before, because every moment counts.
- Implemented milestone-based disbursements. We now have a payment system that rewards timely success and motivates progress.
- Established specific goals and objectives. Our mission is clear, and those responsible for fulfilling it embrace it with conviction. Each team within CIRM establishes quarterly goals that are essential for the overall organization's success. Challenges are quickly identified and corrected. Wins are enthusiastically celebrated.

With each day CIRM gets better and closer to bringing life-changing stem cell treatments to the people who need them the most.

Five-Year Strategic Goals

The Power of Clarity

Diagnosed with retinitis pigmentosa (RP), a rare degenerative eye disease that causes severe vision loss and ultimately blindness, Rosie had retinal progenitor cells—the kind that are destroyed by RP—injected into one eye. Over the course of several months her vision in the treated eye improved.

"My earliest memory is being a clumsy child. I was always falling… tripping, spilling things. I wasn't aware that I couldn't see… I just assumed everyone had the same vision. By the age of six I wore my first pair of eye glasses.

I didn't know I had RP until the diagnosis at the age of 26, when I was pregnant with twins. At the time I learned the disease would lead to blindness and there was no cure. It was devastating news.

Before the stem cell injection my eyesight was 20/400 and progressively degenerating; there was no significant difference between my eyes. After the injection, my treated eye has improved to 20/200. I was surprised to see as much and as quickly.

There's more definition and I'm distinguishing colors I haven't seen in years. I find myself doing more things with my family now that my vision is improving.

My dream was to see my kids. I always saw them with my heart, but now I can see them with my eyes. Seeing their faces… it's truly a miracle."

Rosie Barrero

2002: Diagnosed with retinitis pigmentosa (RP), a rare, degenerative eye disease that causes severe vision loss and ultimately blindness.

2016: BRAVE

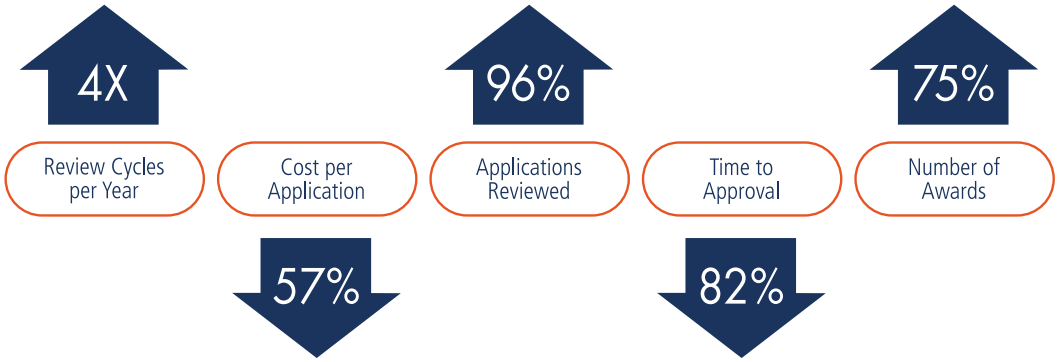
No Time to Lose

Operational Performance Metrics for 2016

A Push for Operational Excellence Is Paying Dividends

Increasing the frequency, speed, and volume of our recurring programs is also leading to significant gains in productivity.

2016 vs. previous five-year average

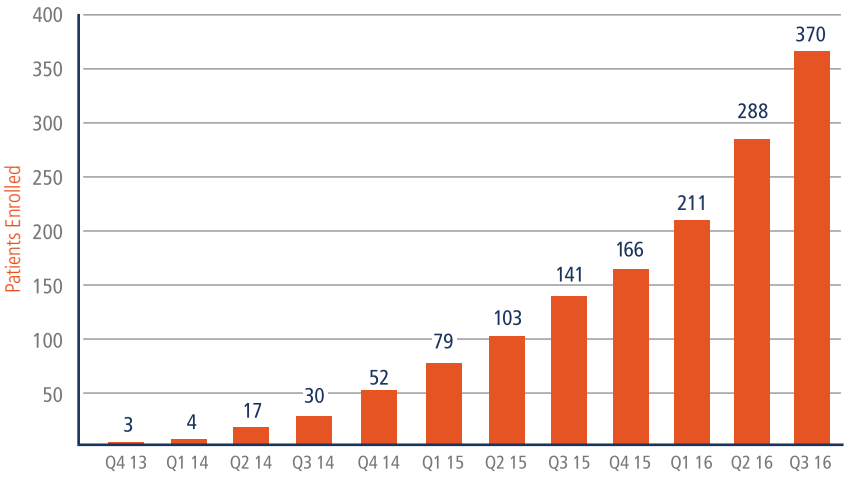


2016 Award Summary

The type, number, and dollar value of 2016 awards

In 2016, CIRM held 22 separate reviews, resulting in 83 new awards that totaled nearly $262 million in aggregate value. Notably, there were 12 clinical stage awards issued. We also made two key investments in our infrastructure, awarding a total of $30 million for the Accelerating Center and Translating Center.

Program	Rounds	Number	Investment
Education	2	21	$43.1 million
Discovery	4	37	$46.7 million
Translation	2	11	$54.7 million
Clinical	12	12	$87.1 million
Infrastructure	2	2	$30.0 million
Totals	**22**	**83**	**$261.6** million



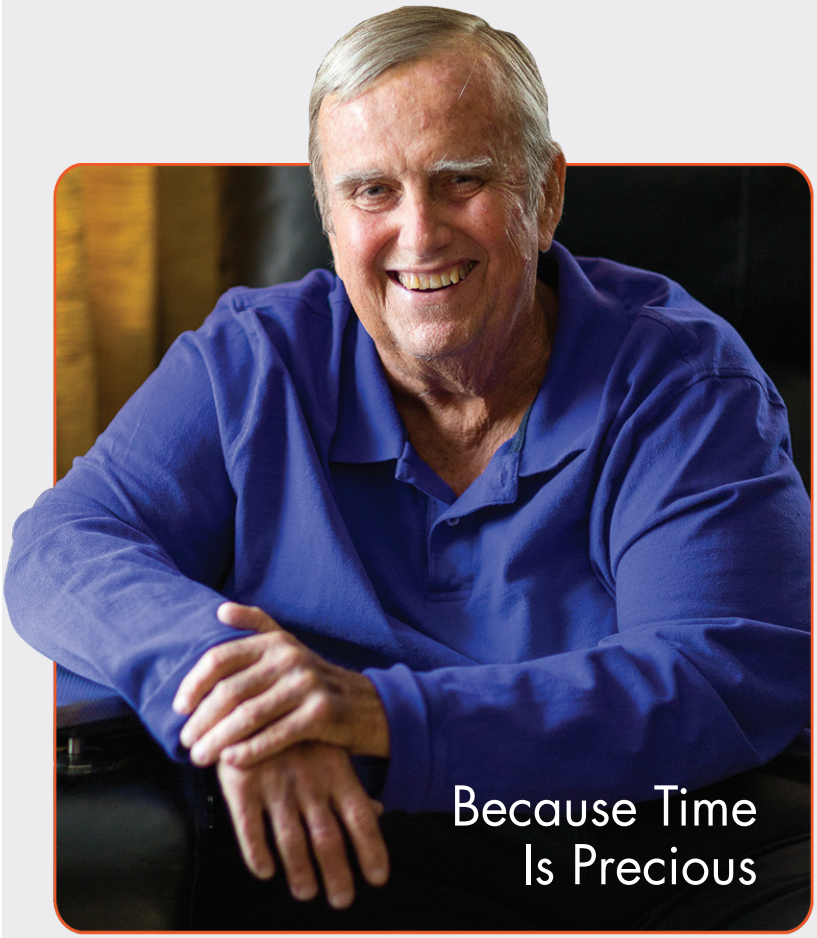
Aggregate Enrollment by Quarter

Patient enrollment rate has increased nearly threefold, meaning a clinical trial that would have taken three years to enroll can now be completed in only 13 months.

Friends,

Our profile of Frank St. Clair is tinged with sadness. Frank passed away on Saturday, December 17, due to complications from heart disease, just a few weeks after this photo was taken. In speaking with his wife Paula, we thought it fitting to leave the profile in this report, unaltered, as a tribute to this courageous and compassionate man, and as a reminder to all of us that time is precious. Frank never gave up and neither will we.

C. Randal Mills, Ph.D.
Chief Executive Officer



Frank St. Clair

Age 63: Diagnosed with kidney disease

Age 67: COMPASSIONATE

Frank has kidney disease and undergoes regular hemodialysis. He recently had a Humacyte acellular device inserted into his arm to make dialysis easier.

Since getting this device, my life has changed completely, 100 percent. When they told me about this I didn't know anything about stem cells. I just knew I was miserable, and if there was any way to make life better I wanted to do it. Participating in this clinical trial was one of the best decisions I've ever made.

I was a private detective. We served a lot of papers on foreclosures. I hated that. I would help people if I could. I ran into an old lady; she was a nice woman, and her husband handled all the bills but he died. They had $1.7 million in stock [in a company that went bankrupt]. She lost it all. When I went to serve her papers she hadn't eaten in two days, so I went and brought some groceries and made sure the electric bill got paid and then called her son and made sure she was taken care of.

My wife said we were going broke helping so many people, but I felt that if you help people it comes back to you, and it has. I got this device and it's unbelievable.

Don't give up. My wife wouldn't let me give up, and good things happen.

> Objective Progress

Milestones Hit on Time

One of our most all-encompassing—and therefore most important—internal metrics is the percentage of milestones our investigators are able to hit on time. It is a measure of both speed and quality, and this year it went up dramatically – due in part to our change to a mile-stone-based disbursement system, where timely success is incentivized. Time matters when lives depend upon it.

Milestones Hit on Time Up Fourfold
19% to 79%

It Was a Strong Year

2016 Results

Established Goals	Delivered Results	
 NEW THERAPEUTICS CANDIDATES DISCOVERED	16	We obviously didn't set the bar high enough, because we achieved this goal, then exceeded it by more than 50 percent. Next year, we step up our game even more.
 PROGRESSION EVENTS	16	We want to fund the most promising science and make sure it successfully progresses to the next stage of development—a Progression Event. This year we did just that.
 ESTABLISH NEW REGULATORY PARADIGM	YES	Getting the FDA, Congress, and the President all to agree on a new approach to stem cell regulation (the 21st Century Cures Act) was not an easy task. But in the end the combined efforts of many different groups—including CIRM—did just that, and on December 13, 2016, the Act was signed into law.
 CREATE STEM CELL TRANSLATING & ACCELERATING CENTERS	YES	This year CIRM combined and opened both Translating and Accelerating centers in a unique way to help the most promising research progress out of the lab and into clinical trials faster.
 NEW CLINICAL TRIALS	10	At midsummer we had only two clinical trials approved, and lowering our standards was not an option. Team Therapeutics (one of the groups charged with this goal) looked to be in trouble. But they staged a dramatic fourth-quarter comeback and achieved their goal in December.
 PARTNERED CLINICAL PROJECTS	2	With two partnerships, we made significant progress but fell short on this goal. We're working to complete the job and secure the third partnership in 2017.

BIG 6 2016

And we're getting stronger

#JakeStrong

An accident on the eve of his high school graduation left Jake paralyzed from the chest down.

Following a stem cell treatment, he regained some use of his arms. Jake hopes the stem cells will provide the return function of his hands which he has completely lost, as well as his triceps. He presently uses a wheelchair for mobility. Prior to his accident, he was heading to Cal Poly University in San Luis Obispo to play football and study mechanical engineering.

"I like to be involved with sports and outdoor activities. While at Craig Hospital in Colorado, we went on several hikes and many outdoor activities such as fishing and hunting. Some activities did require adaptive devices due to my disability which Craig provided. We also took a family day trip to Rocky Mountain National Park, the scenery was stunning. Participating in outdoor activities has definitely played a vital role in my recovery.

I attended a wheelchair rugby clinic while at Craig. I was transferred into a rugby wheelchair and had an opportunity to practice with some of the Paralympic players. I instantly liked it as it was fast paced and exciting.

Next fall, it is my desire to attend Cal Poly to pursue my mechanical engineering degree. I will try and stay involved with the football program by assisting the coaches to the best of my ability."

Jake Javier

Age 18: Sustained a spinal cord injury, leaving him paralyzed from the chest down

Age 19: STRONG

Planning and Preparing for Success

2016 Financial Reconciliation

Award activity for calendar year 2016

Our strategic plan's fiscal projections remain on track. During the year the uncommitted balance (the amount of money remaining that has not been earmarked for a specific project) dropped from $760 million to $528 million. This was the result of $262 million in new awards being issued, offset by $30 million in active award reductions. CIRM currently has $406 million under active management, and projects that $692 million will be made available for new awards through mid-2020.

As of January 1, 2016	
• Committed / Uncommitted balance	**$1.99 billion / $760 million**
• Number / Balance under active management	**261 / $342 million**
2016 Activity	
• Number / Amount of awards added	**84 / $262 million**
• Award reductions	**$30 million**
As of December 31, 2016	
• Committed / Uncommitted	**$2.22 billion / $528 million**
• Number / Amount of active management	**255 / $406 million**
Projected funding available for new awards	**$692 million**

Continuous Improvement

Above all, we want to accomplish our mission. When we launched CIRM 2.0 on January 1, 2015, we set out to radically change the way we fund stem cell research, to speed up our process and make it easier for the most promising projects to get funding fast. Since then we have made a number of improvements in our system. And we will continue to do so in the years to come. We must continually and objectively assess our progress and make timely changes where needed. A few of the changes we have made to further refine and improve the system:

- Revised the scoring system to provide clarity to the review recommendations
- Added a process to evaluate the rigor and fairness of the review
- Made past performance information available to reviewers
- Eliminated indirect costs for for-profit institutions
- Established project milestones for CIRM's Discovery Program and Translation Program

Approved Budget for Calendar Year 2017

The type, number, and dollar value of budgeted 2017 awards. (Rounds and awards are for guidance purposes and non-binding.)

The board-approved budget for 2017 calls for $329 million in new investment, which approximates to 70 new awards being issued in 20 separate rounds. This budget includes $16 million for the creation of two new Alpha Clinics.

Program	Rounds	Number	Investment
Education	N/A	N/A	$1 million
Discovery	4	40	$52 million
Translation	3	9–12	$45 million
Clinical	12	up to 17	up to $215 million
Infrastructure	1	2	$16 million
Totals	**20**	**TBD**	**$329** million

We will never stop getting better

ALL IN. ALL OUT.

Risk Review

The poet T. S. Eliot said, "Only those who will risk going too far can possibly find out how far one can go." At CIRM we know that there is risk in almost everything we do, but we also know that to settle for anything less than the best would mean failing to live up to our mission.

The biggest risks we face are:

- Not enough high-quality applications for us to reach our goals. This has proved not to be the case in 2016, but we know we have to work hard to ensure that we continue to attract the very best science.
- Not enough interest from qualified applicants to participate in key competitions. Our Translating Center and Accelerating Center awards attracted some high-quality applicants, helping us successfully launch those programs this year. But we ended with no qualified application for our Accelerated Therapies Public Private Partnership (ATP3) program.
- Investors may be uninterested in stem cell treatments. The lack of a track record of success, and the problems posed by an overly burdensome regulatory process, mean that the venture capital, big pharma, and biotech sectors still are reluctant about making substantial investments in stem cell research. Again, this risk was evident in our ATP3 process.
- Stem cell treatments may not provide sufficient benefit to create enduring demand. If stem cell treatments fail to show that they are better than the currently available therapies, that could lead to a lack of demand for, and investment in, stem cell treatments.
- Safety concerns may arise that slow or limit the ability to conduct clinical trials. One serious setback—a patient death, for example—can have an enormous impact on the field as a whole, raising questions about safety and halting clinical trials. We are committed to conducting clinical trials to the highest scientific standards and taking prompt action should any problems occur.
- We must always be cognizant of the risk of losing members of our excellent team. They are the reason the organization has performed so well, and they are essential to CIRM reaching its goals in the future. Simply stated, they are the best, and we need them to stay with us for as long as possible.

Karl Trede

Age 60: Diagnosed with throat cancer, then later lung cancer, for which there was no effective treatment

Age 70: COURAGEOUS

"In 2006 I was diagnosed with cancer of the throat, and had the tumor and my vocal cords removed. Several years later they found the cancer had spread to my lungs.

My doctor calls me a pioneer. He said we know that there's no effective treatment for this cancer; it's not likely, but it's possible that this could be the one. If nothing else, if it doesn't do anything for me, hopefully it does something so they learn for others.

I consider myself very fortunate. I'm 70 years old and still doing well.

I have four sons and we spend a lot of time together. We do a lot of cooking together. Last year we decided to have a rib cook-off for 30 people. I can proudly say that I kicked their butts in the rib cook-off. I have an electric cooker and I just cook 'em slow and long. I'm only able to do that, spend quality time with them, because I am still here."

A 2020 VISION

Performance Against CIRM's Five-Year Goals

Discover

Introduce 50 new candidates into development

Rationale: It all starts with discovery. Today's new concepts are tomorrow's cures, so we want to make sure our engine is well fueled with a healthy supply of fresh new ideas.

Status: 16 new candidates (32 percent to our goal). We are well on our way with this goal and should reach 50 new candidates in early 2019, well ahead of schedule.

Advance

Increase projects advancing to the next stage of development by 50 percent

Rationale: When a project makes the jump to the next phase of development—for example, from discovery to translation—we say a Progression Event (PE) has occurred. PEs are a big deal at CIRM because they show that the project and CIRM's process are working well.

Status: Up 33 percent. We are already two-thirds of the way to our goal and are well positioned to exceed the 50 percent mark by 2020.

Refine

Enact a new, more efficient regulatory paradigm for cell therapies

Rationale: It's been over 15 years with the current regulatory framework, and the FDA has approved no stem cell product. Part (but certainly not all) of the reason is the system.

Status: On December 13, 2016, following overwhelming bipartisan support in both houses of Congress, the 21st Century Cures Act was signed into law by the President. It provides an accelerated approval pathway for stem cell treatments for serious conditions. Only time will tell if it is truly effective, but it's a great start.

Accelerate

Reduce translation time (discovery to clinical trial) by 50 percent

Rationale: It is taking about eight years for stem cell candidates to progress to the phase where human clinical trials can begin. For non-cell therapies, that same journey takes only 3.2 years. We are looking to cut translation time for cell therapies in half.

Status: To evaluate this one, we break it into the two CIRM programs that cover translation time, the Translation program (TRAN1) and the Investigational New Drug enabling program (CLIN1). If the median times for the TRAN1 and CLIN1 programs are less than 2.5 and 1.5 years respectively, we will have hit our goal. Neither program is far enough along yet to establish a median time; however, our "milestone hit on time" metric indicates things are going well, and the addition of the new Translating and Accelerating Centers should really boost this one.

Validate

Add 50 new clinical trials to the CIRM portfolio

Rationale: Clinical trials are the final step in the development process, yet only one in 10 treatments that enter human testing are ultimately approved. With those odds, we need multiple shots on goal.

Status: Ten new trials or 20 percent to goal. After a slow start to the year, CIRM's Therapeutics team has really turned it around, placing us right on track.

Partner

Pair 50 percent of unpartnered clinical projects with commercial partners

Rationale: To fully realize our mission and create long-term benefits for the people of California, we need to engage industry.

Status: Two new partnerships were created this year. We have also realized an uptick in industry involvement with academic researchers prior to application to CIRM.

At CIRM Every Moment Counts

"She's spunky and a fighter. I think of her as a normal little girl that was born in extraordinary circumstances and had extraordinary doctors cure her, which is amazing."

Born with SCID or "bubble baby disease," Evangelina had no functioning immune system, leaving her vulnerable to infections.

As part of a clinical trial at UCLA, she underwent a stem cell transplant that took her own blood stem cells, genetically re-engineered them, and returned them to her body. These re-engineered stem cells rebuilt Evangelina's immune system.

Her parents knew this was a trial and might not be a cure, that it was an experiment. They believed they had no other options but this one.

The treatment worked. Slowly, steadily, Evangelina got well, got stronger. Her mother said:

"Evangelina was around 2 when we finally got up the nerve to take her to public places. We'd spent so much time worrying about her being exposed to germs that it took a while to get un-used to it. The tipping point was taking her to Disneyland when she and her twin were 3 years old. We let them touch everything. My husband and I were dying inside but they had a great time. It's been amazing. I look at her every single day and think, 'She's a miracle.'

Evie is my little genetically modified supergirl."

Evangelina Padilla Vaccaro

2012: Diagnosed at birth with severe combined immunodeficiency (SCID), a rare—and often deadly—genetic disorder

2016: CURED

Our ALL IN Culture

At CIRM we work every day as if lives depend on it, because they do. Every member of our team, from the board to our expert reviewers, has the same dedication to our mission, that same commitment to doing all we can to help patients. From time to time we honor and celebrate those who exemplify that commitment and reflect the urgency and passion of our world-class team with a "CIRM Game Ball."

2016 CIRM Game Ball Winners

Kent Fitzgerald—for single-handedly helping move a Discovery research project progress to a Translation one.

Doug Kearney—for launching the first six CIRM 2.0 award contracts in record time.

Jennifer Mielnicki—for hitting a seemingly impossible contract milestone that got research under way sooner than we thought possible.

Kevin McCormack—for always being willing to say "yes, absolutely" no matter what the challenge.

Karen Ring—for creating the score-cards that give us a highly visual reminder of what our goals are, and how close we are to hitting them.

Sheila Tennyson (a two-time recipient)—for making CIRM's move from San Francisco to Oakland smooth and painless, and for her dogged determination to be a good public speaker.

Rahul Thakar—for developing a more efficient way of writing grant review summaries, enabling applicants to get feedback faster and make adjustments to re-apply as quickly as possible.

RELENTLESS

Pat Furlong
Patient Advocate

2016 CIRM Game Ball Winner

Pat Furlong is the founding president and CEO of Parent Project Muscular Dystrophy, the largest nonprofit organization in the United States solely focused on Duchenne muscular dystrophy.

Their goal is simple: Change the course of Duchenne and, ultimately, find a cure.

Duchenne muscular dystrophy is the most common fatal genetic childhood disorder. It's a progressive muscle disorder that leads to loss of function in the body's muscles, including the heart. There is no cure.

When doctors diagnosed her two sons, Christopher and Patrick, with Duchenne in 1984, Pat didn't accept "there's no hope and little help" as an answer. Pat immersed herself in Duchenne, working to understand the pathology of the disorder, the extent of research investment, and the mechanisms for optimal care.

Her sons lost their battle with Duchenne in their teenage years, but she continues to fight—in their honor and for all families affected by Duchenne.

Today, CIRM is funding a clinical trial using heart stem cells to treat heart problems caused by Duchenne muscular dystrophy.

> *"For me, staying in this fight is to be able to say to Chris and Pat, wherever they are in the universe, when you were here I tried my very best, and when you were gone I continued to try my best, so that others would have advantages that you didn't receive."*

Our ALL OUT Culture

Investing in the next generation of stem cell scientists creates a trained workforce for California and ensures that the state has the talent available to continue the search for cures. Altogether, we've helped 2,432 people pursue their dreams—dreams that would otherwise not have come true—as well as supported dozens of young faculty members as they establish their careers in California. At CIRM, we are going ALL OUT to make our mission a reality.

High School Students

High school students interested in a career in science can get a head start on their dreams with SPARK, short for the Summer Program to Accelerate Regenerative medicine Knowledge. We support high school students throughout California in stem cell research summer internships in research labs at prominent California institutions. In past years, we have asked those students to show us the value of their experiences in blogs, videos, and images.

Undergraduate and Master's Students

For our undergraduate and master's-level students, Bridges to Stem Cell Research Awards provide coursework and internships at California state schools and community colleges, followed by paid internships at the state's top university and industry stem cell labs. These programs train young scientists to fill jobs in California's growing stem cell research sector, filling a void predicted by both the California Life Sciences Association and the California Public Policy Institute. The programs' first graduates are already being hired into skilled research technician positions and being accepted into medical and graduate schools in large numbers.

Graduate and Postdoctoral Students

Our first round of funding in 2006 was to support training grants to make sure California had scientists with the training needed to develop stem cell–based treatments. These awards supported graduate students and postgraduate students carrying out stem cell research in California institutions. Many of our training grant recipients have gone on to start their own stem cell research labs as faculty members and are carrying forward advances for the next generation of stem cell treatments.

New Faculty Awards

Although their formal education is over, new faculty members need support while they are starting up their labs. CIRM has provided a stable source of funding for some of the best young faculty members through our New Faculty Awards, which have helped them establish their stem cell research programs in California.

Discovery Day

Part of a week-long free public science festival that draws tens of thousands of attendees. CIRM uses interactive activities to explain stem cell biology to children of all ages, taking the opportunity to talk to their parents about the progress being made by CIRM in advancing the field of regenerative medicine.

Super Stem Cells Exhibit

An award-winning high-tech interactive exhibit that helps teach children aged 6 through 14 (as well as their parents) about stem cell biology and the potential it has to transform medicine.

Roadshow

A series of events held around California to inform industry and academia about the funding opportunities at CIRM. Designed to "reintroduce" CIRM—and all that the stem cell agency can do—including partnering with researchers to give them the best possible chance of succeeding.

Alpha Stem Cell Clinics Network

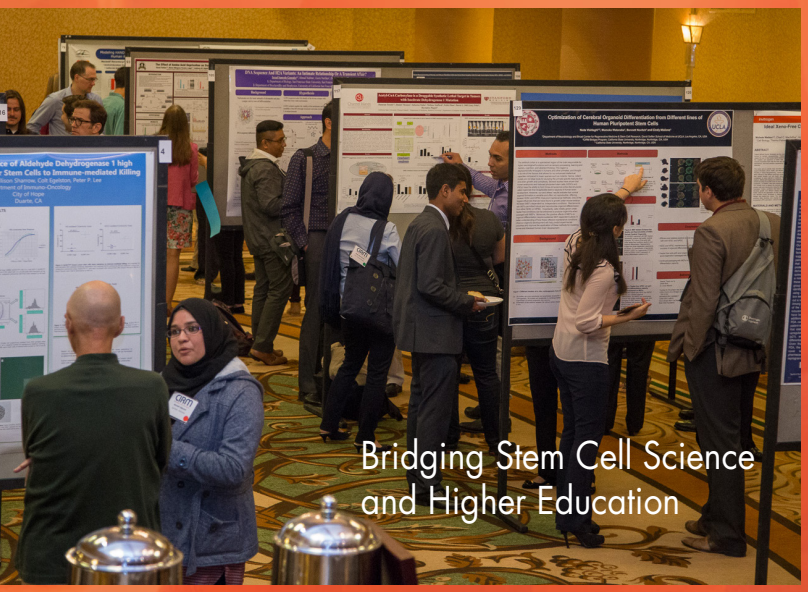
The Alpha Stem Cell Clinics Network has one unifying goal: to accelerate the development and delivery of stem cell treatments to patients. To achieve this goal, CIRM has funded three stem cell–focused clinics, housed within existing medical centers. This network of clinics will attract and conduct high-quality trials:

- Together with patients
- In collaboration with national and international researchers
- In partnership with academia and industry
- Within top-notch academic medical centers
- With the highest standards for patient care and research
- With resources that promote efficiency, acceleration, and chances of success

The key to the Alpha Stem Cell Clinics Network's success is its ability to leverage the strengths of each individual clinic—so that together they form an efficient and sustainable network that will attract and conduct high-quality clinical trials. The clinics support both CIRM-funded and non-CIRM-funded clinical trials.


Training Tomorrow's Scientists
CIRM
CALIFORNIA'S STEM CELL AGENCY


Discovery Day


Bridging Stem Cell Science and Higher Education


ALS Walkathon
STEM CELL CHAMPION
Social Media Volunteer


Super Stem Cells Exhibit

OUR RESOLVE

is strong. Our purpose clear.

We are driven.

All In. All Out.

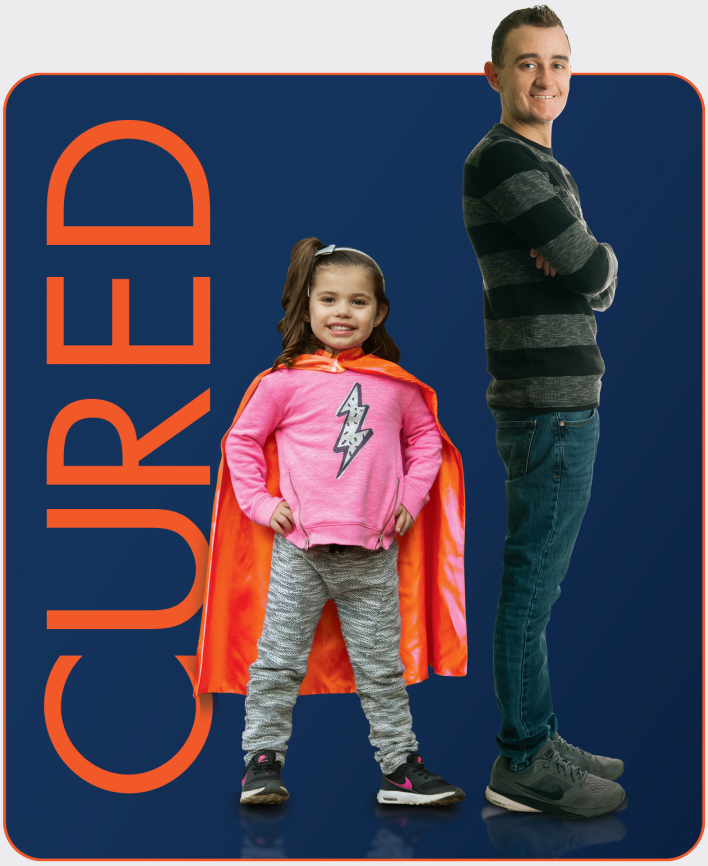
To do more. Faster. Better.

To achieve all that is possible,

not for ourselves but for those

who need us the most.

Every Moment *Counts*



Courage and Vision Realized

Evangelina Padilla Vaccaro
Previously diagnosed with severe combined immunodeficiency disease

Brenden Whittaker
Previously diagnosed with severe chronic granulomatous disease

This year, there could be no better example of realizing CIRM's vision than the incredible success stories of Evangelina and Brenden, both of whom suffered previously incurable diseases. Their cures made possible by the team led by Dr. Donald Kohn, renowned stem cell researcher and professor of pediatrics at the UCLA David Geffen School of Medicine.

Evangelina's clinical program was born out of a CIRM educational funding grant, and Brenden's treatment was funded through a clinical trial grant. These two success stories serve as powerful bookends that represent the impact of leveraging the full potential of CIRM's investment programs for meeting unmet medical needs.

That's why we at CIRM are *all in*, and going *all out*.
Because every moment counts.

CIRM
CALIFORNIA'S STEM CELL AGENCY

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