

PERSEVERANCE

from theory to therapy

Sneha Santosh
Former Bridges Student
and Researcher

Catriona Jamieson
Physician and
University Scientist

Byron Jenkins
Patient

Ysabel Duron
Patient Advocate

Mark Chao
Physician and Biotech
Researcher



Evie Padilla Vaccaro
Patient

CIRM
CALIFORNIA'S STEM CELL AGENCY

2019-2020 18-Month Report

Living Our Mission

We work with focus and urgency, as though someone's life depends on it. Because it does.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs. We act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast-track the development of today's most promising stem cell therapies — right here in California.

With \$3 billion in funding and more than 1,000 stem cell programs in our portfolio, CIRM is one of the world's largest institutions dedicated to helping people by bringing the future of cellular medicine closer to reality.

California Proposition 71

Paving the way for a new era of medicine, California Proposition 71 provides us with the opportunity to fund long-term projects, keep California-based scientific teams together, identify the most promising stem cell research, and support and accelerate that research into therapies to meet the health needs of Californians — and the world.

CIRM's work has proven to be an important lever for securing additional funding from investors for California-based research that may not have come to our state any other way.

Accelerate stem cell treatments
to patients with unmet medical needs

Dear Fellow Californians,



Welcome to our look back at the last 18 months at CIRM that provides an up-to-date view of our accomplishments and all that California has achieved in establishing a global hub of the emerging regenerative medicine industry right here in our state.

Our work in accelerating the discovery and development of a new field of medicine not only brings treatments and cures to patients with serious and life-threatening conditions, it also benefits California as a whole.

An independent Economic Impact Report shows that the state — led by CIRM's efforts — is generating billions of dollars in new sales and tax revenues and creating tens of thousands of new jobs. In addition, our education programs are developing a top-notch workforce to fill the pipeline of jobs in California's growing number of regenerative medicine companies.

CIRM accelerates the development of good science, including emergency-action projects to address COVID-19 in California, and makes breakthrough therapies available to its citizens through the statewide Alpha Stem Cell Clinics Network. We also take a lead role in raising awareness about predatory clinics that offer unproven "treatments" that put patients' health and lives at risk, while informing people about high quality, scientifically sound therapies.

Our commitment, as always, is to serve patients in need. We are confident that the world-class work we have enabled will do just that.



Jonathan Thomas, Ph.D., J.D.
Chairman, CIRM Governing Board

Dear Friends,

In the past 18 months, CIRM has supported 14 more clinical trials (64 total at last count). When the coronavirus pandemic hit, the CIRM funding model gave us the flexibility to respond by quickly supporting research into COVID-19, including three clinical trials. We are also testing therapies developed right here in California to treat cancer, diabetes, sickle cell disease, and other serious diseases and conditions.

Compiling scientific evidence takes time, but CIRM has accelerated the pace at which California's regenerative medicine research and discovery advances and is made available to patients throughout the state.

The path to get a therapy approved by the Food and Drug Administration (FDA) can take 12 to 15 years, require thousands of patients for clinical trials and cost billions of dollars. CIRM is helping overcome those hurdles, working in partnership with the FDA to create a streamlined approval process that delivers much-needed treatments to patients who have few, if any, options to improve their debilitating or fatal conditions.

One thing that makes CIRM unique is our ability to identify, vet and support promising high risk but high reward science at the discovery stage supporting projects that may otherwise languish due to lack of funding—the phase of research that drives innovation, but very rarely draws commercial investment. As featured in this report, CIRM has provided critical support for early-stage scientific discoveries at academic institutions that have gone on to secure commercial partnerships and investments that are helping move treatments closer to patients.

As you'll see in this Annual Report the perseverance of CIRM's leadership, the researchers we fund and the patient advocate community we serve is building something to last.



Maria T. Millan, M.D.
President and Chief Executive Officer



Advancing Science

Accelerating Progress
Aligning Opportunity

A wholly new ecosystem for advancing regenerative medicine

In the United States, it takes an average of 12 to 15 years for a typical new chemical drug to progress from the laboratory to a patient. Even then, out of 5,000 new drugs being developed, only five will advance to clinical trials, with just one gaining FDA approval. The cost of bringing a single drug to market is said to be more than \$1 billion.

In California, 64 CIRM-supported programs to find new medical therapies (not chemical drugs, but therapies created from the body's own cells) have advanced to clinical trials. CIRM also funded another 30 early-stage projects that later advanced to the clinical stage of development, including two that have been approved by the FDA. These therapies emerged from the completely new field of medical science built on stem cell and gene-based therapies enabled by CIRM.

Of the CIRM-supported clinical trials, four are in the final stage before FDA approval (Phase III), as of June 2020.

Unlike traditional funders of medical research, CIRM has developed a systems approach to discovering and accelerating innovative medical treatments and has created a new, growing, multidisciplinary field of medical science—regenerative medicine—in California.

CIRM is credited with creating California's robust research, development, and clinical trial pipeline by doing things differently.



Accelerating Progress

De-risking innovation by funding early research (or “bench” science)—the stage of research that is the foundation of innovation, yet often overlooked by other funding sources

Vetting grant applications with rigor and incorporating input from patient communities, as well as insights from experts in their fields

Structuring financial awards that are dependent on meeting certain scientific goals and milestones

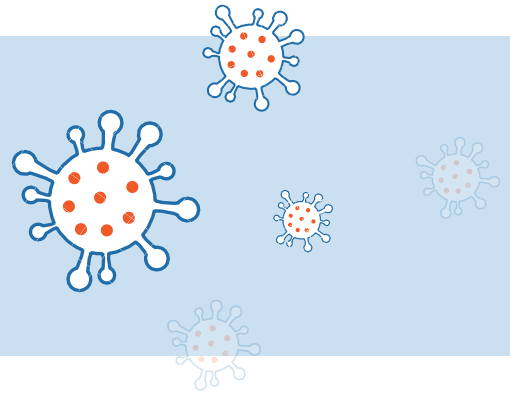
Matching grantees with an advisory panel that helps accelerate researchers' progress and brings experience, knowledge, and resources to overcome obstacles and to help prepare for and navigate clinical trials

Supporting workforce development to prepare young people in California for careers in regenerative medicine

Making clinical trials at world-class medical centers available to the people of California through our funded institutions and CIRM's statewide network of Alpha Stem Cell Clinics

ADDRESSING COVID-19

Because Every Moment Counts



The sooner we can get support to researchers, the sooner they can deliver the therapies that could save lives. CIRM's operating model has become the gold standard for fast funding and acceleration of medical research, but in the face of the COVID-19 crisis we knew we had to go a step further. We created a special \$5 million emergency fund for rapid research to address the pandemic and significantly shortened the time that a grant request moved from application to funding to four weeks, rather than four months.

Within a few weeks of approving the funding, we were supporting a clinical trial to determine if blood plasma from patients who had recovered from the virus could help those newly infected. Another trial is designed to mitigate the damage the virus causes to lungs.

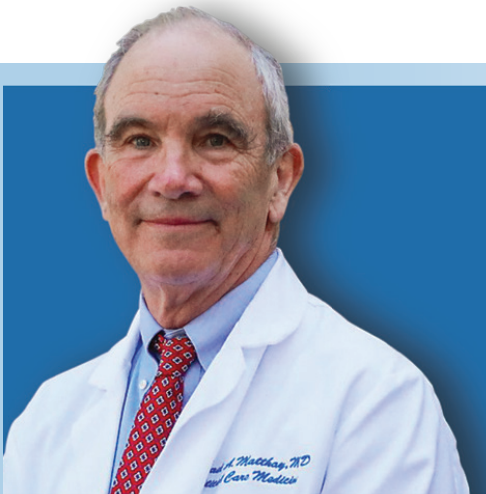
Extraordinary times require extraordinary action, and CIRM and the State of California are at the forefront of these efforts.

Our CIRM Alpha Stem Cell Clinics Network—with five clinics across the state—quickly expanded its efforts to respond to the coronavirus by:

Enrolling patients in clinical trials testing potential therapies for COVID-19

Expanding telemedicine access to communicate with trial patients without them having to travel to the medical center

Developing stem cell models in the lab to test potential vaccines and medications before trying them on people



Michael Matthay, M.D.
University of California, San Francisco

CIRM funding is helping Dr. Michael Matthay at the University of California, San Francisco, expand a clinical trial to help underserved communities. People of color have been disproportionately affected by the outbreak of COVID-19, yet they are typically not well represented in many clinical trials. Dr. Matthay and his fellow researchers are changing that—making sure potential treatments benefit all Californians, without regard to income, insurance status or ethnicity.

One of the deadliest aspects of COVID-19 is the way it attacks the lungs. Even for patients who survive respiratory failure, the path back to normal daily living and work can take up to one year.

Dr. Matthay is running a clinical trial using stem cells to treat people with acute respiratory distress syndrome (ARDS), a serious form of respiratory failure found in many patients with severe cases of COVID-19. He explains, "This stem cell therapy is designed to help the lungs repair themselves, get patients off ventilators, and increase patients' odds of survival."



CIRM-Funded **Clinical Trial**

Following the Science: from California to the world

Transforming knowledge, discovery and science into innovation

Significant and sustained investment—led by CIRM, along with other funders—follows the science.

The science starts with basic research and discovery of how stem cells work. This early stage of research is where breakthrough therapies take shape, but because ideas are not yet proven, funders can be reluctant to invest. CIRM’s approach is different. CIRM has systems in place that help identify and support the most promising science at the earliest stages of development, clearing the path, and inspiring other funders to get involved.

In the next phase, researchers take these new ideas and discoveries and translate them into potential therapies, designed to solve specific medical problems. Finally, only the most promising are tested in clinical trials.

At every step along the way, CIRM partners with the researchers—bringing in outside experts as necessary—to help them advance and succeed.

A new industry follows new science

Regenerative medicine is flourishing in California. Research continues to accelerate at a rapid pace, the number of biotechs in the state is continuing to grow, funding from a wide variety of sources is following CIRM’s lead, and the number of clinical trials in the FDA-approval pipeline has exponentially increased.

“ We’re going to see more and more products reach market...we are on this essentially very steep portion of a growth curve.”

Dr. Peter Marks of the FDA Center for Biologics Evaluation and Research at a 2019 meeting of the Alliance for Regenerative Medicine



The scientist who wouldn't take no for an answer

Catriona Jamieson, M.D., Ph.D.
University of California, San Diego



CIRM-Funded Researcher

Being a great scientist is not just about having a brilliant mind, it's also about being persistent. Dr. Catriona Jamieson embodies both these qualities. The University of California, San Diego, physician/researcher and current CIRM Alpha Clinics director championed early research, which was supported by CIRM and led to the development of a therapy called fedratinib to treat myelofibrosis, a type of blood cancer originating in the bone marrow.

PERSISTENT

During clinical trials, the company that owned the rights to fedratinib halted testing—when the drug was very close to regulatory approval—after one patient's death. While the therapy proved effective in patients, helping many who had no other treatment options, U.S. regulators put a safety hold on the medication.

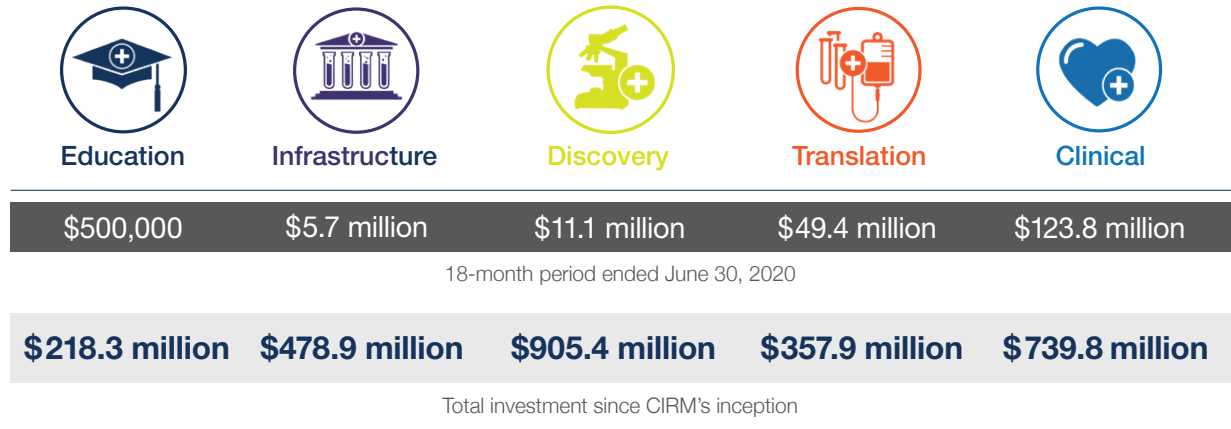
Dr. Jamieson felt this therapy was too promising to be sidelined. She fought to continue the drug's advancement.

Dr. Jamieson went on to co-found a company that resumed fedratinib's development and led the research needed to show it was not just safe, but also effective.

In August 2019, the FDA approved the drug, now marketed as INREBIC®, the first new treatment for myelofibrosis in a decade.

2019-2020 Strategic Investments

CIRM supports education and research at various levels of development, from building infrastructure to clinical trials, that collectively form a unique regenerative medicine “ecosystem” in California.



Education

CIRM is helping California prepare for the future by inspiring and guiding the next generation of scientists. We invest in students and young people, creating internships, educational programs, and training opportunities that give them the skills they need for a career in California’s emerging regenerative medicine industry.

Infrastructure

CIRM helped create 12 state-of-the-art laboratories that have attracted some of the best scientists in the world to California. We developed a network of top class medical centers to deliver treatments to patients and a stem cell research bank that is now the largest of its kind in the world.

Discovery

Every great invention begins with a simple idea, someone daring to pose the question that starts with, “I wonder if.” CIRM shares that sense of wonder and curiosity. Discovery—basic level research—is the key to innovation and at the heart of everything CIRM does. New ideas that push the boundaries of science and the scientists who dare to ask those “what if” questions is how science advances.

Translational

CIRM’s goal is to take every idea that shows potential and help support and develop it, from an interesting theory to a promising therapy. Translational research does that, building on what’s learned in Discovery then pushing it on to the next stage, clinical trials, where we see if it really works.

Clinical

Everything CIRM does is focused on getting therapies to patients. Clinical trials—where new therapies are tested in people—are a vital final step in that process. It can take years of dedication, hard work and rigorous testing to see a project through from Discovery to a clinical trial. Only the very best projects get this far, and to date, CIRM has funded 64 clinical trials. Another 30 projects in which CIRM provided early support have also gone to clinical trials—including two that have been approved by the FDA.

TOP GUN



Byron Jenkins

Patient



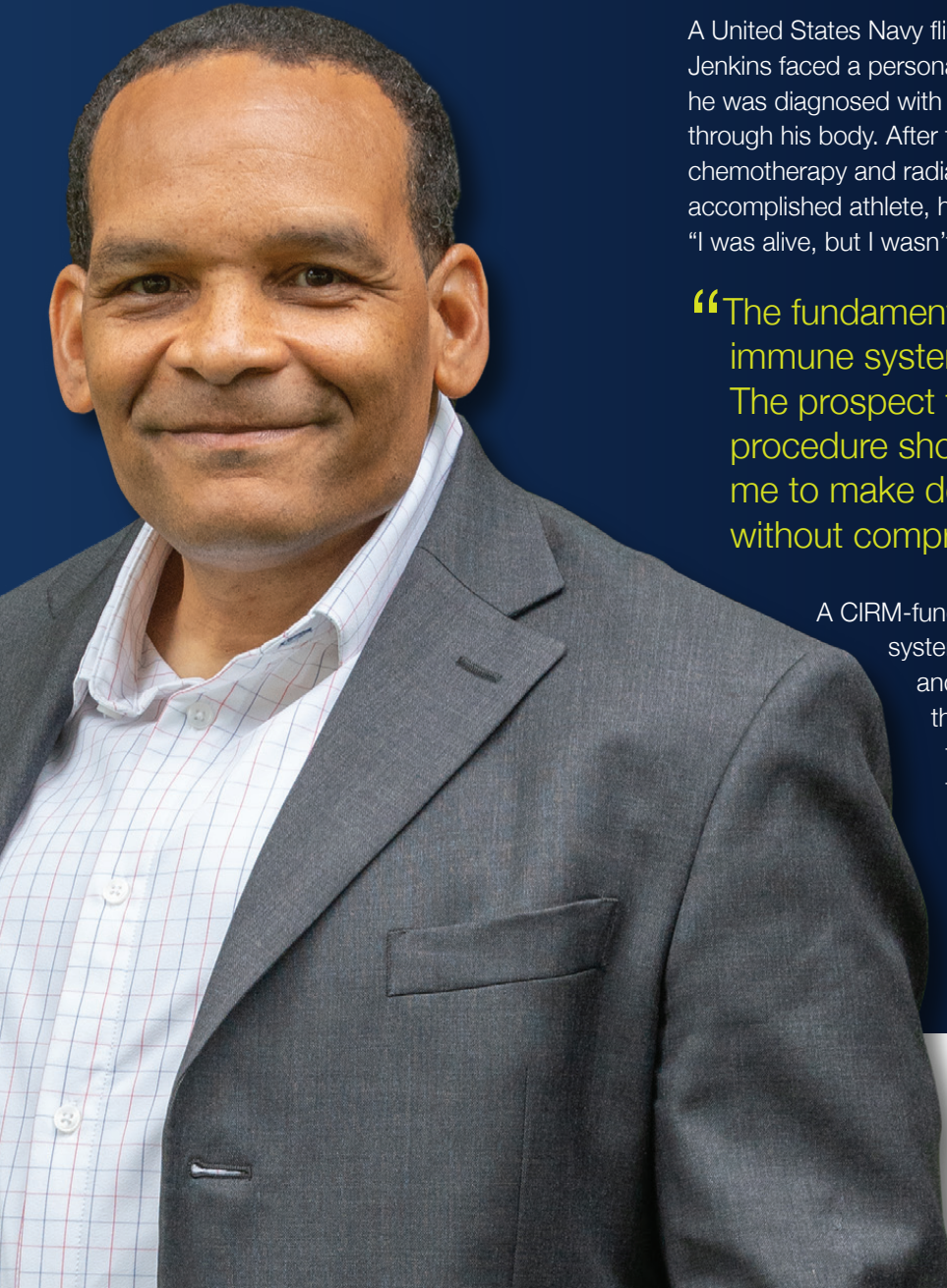
CIRM-Funded **Clinical Trial**

A United States Navy flight officer and warfare weapons expert, Byron Jenkins faced a personal deadly foe: blood cancer. In June 2013, he was diagnosed with multiple myeloma, a blood cancer coursing through his body. After two years of traditional treatment—chemotherapy and radiation—he remained bedridden. Once an accomplished athlete, he was barely able to move. He was in despair. “I was alive, but I wasn’t living,” he said.

“The fundamental concept of letting my own immune system fight the disease comforted me. The prospect that the theoretical nature of this procedure should never stop working allows me to make decisions in my life without compromise.”

A CIRM-funded CAR-T therapy took Byron’s own immune system cells (T-cells), re-engineered them in the lab, and weaponized them to recognize and neutralize the cancer cells. Putting that into naval aviation terms, T-cells are like an F-14 Tomcat: built to fight—and win.

Within weeks of the treatment, Byron was off all medications. Today, he is flying high and enjoying a full, active life with his family.




5-Year Goals The Big Six

Results as of June 30, 2020



DISCOVER Status **46** candidates

Introduce 50 new treatment candidates into development




ADVANCE Status **100%** increase

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



REFINE Status **6** CIRM-funded therapies qualified for FDA accelerated review (RMAT)

Enact a new, more efficient regulatory paradigm for cell therapies



ACCELERATE Status **72%** candidates achieved IND within 24 months

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



VALIDATE Status **47** trials added in five years (64 total)

Add 50 new clinical trials to the CIRM portfolio



PARTNER Status **59%** partnered

Pair 50% of unpartnered clinical projects with commercial partners

In 2015, CIRM laid out a bold, strategic plan with ambitious goals, charting a course for the next five years. As we head into the final stretch of the plan, goals that once seemed audacious have already been met, and others are now within reach.

Over the past five years, CIRM has created a better, faster, more efficient system for advancing promising stem cell and gene therapy science. By creating a model for accelerating regenerative medicine, CIRM is quickly reviewing and approving grant applications, speeding up funding for successful applicants, creating new pathways for CIRM-funded researchers to have continued success with commercial partners, and helping prospective therapies vault regulatory hurdles expeditiously.

For example, before 2015, the time it took to move a project from application to funding was more than a year. Today, it takes about four months. With the unprecedented rapid onset of the coronavirus pandemic and the need for even greater urgency in getting projects off the ground, CIRM expedited the grant approval process even further, to around four weeks.

We never stop looking for ways to improve, to be faster, smarter, and more responsive. Why? Because that's why we are here, to accelerate stem cell treatments to patients with unmet medical needs.

64 Clinical Trials Funded

CIRM doesn't just fund stem cell research, we support it at every step along the way.

CIRM partners with patients and patient advocates to bring their knowledge and expertise to our work, helping guide and shape the projects we invest in.

Firmly Rooted
in California

5 Alpha Stem Cell Clinics located throughout California created to deliver stem cell therapies to patients in need

6 projects given Regenerative Medicine Advanced Therapy (RMAT) designation by the FDA, potentially making them eligible for faster review and approval

12 world-class stem cell research laboratories funded to advance the field in California

14 projects that are in late-stage preclinical testing, the last step before applying to the FDA for permission to start a clinical trial

30 projects where we provided funding for early-stage research have gone on to be tested in clinical trials

>40 children born with Severe Combined Immunodeficiency (SCID), a fatal immune disorder, cured

64 clinical trials for COVID-19, cancer, stroke, heart disease, diabetes, vision loss, sickle cell disease, HIV/AIDS, spinal cord injury, leukemia, immune disorders, kidney failure, arthritis, Parkinson's disease, and more

>1,000 projects from early-stage discovery research to clinical trials

>2,000 patients enrolled in CIRM-funded clinical trials

Delivering on our Mission

Science drives investment

An emerging industry creates an economic catalyst

With both the health and economic well-being of the state in mind, CIRM is supporting transformative treatments that can bring new therapies to patients as it also builds the world's leading innovation hub for stem cell research that is attracting financial investment, industry, and top talent to the state.

CIRM's efforts have stimulated growth of the regenerative medicine industry in California. The state's rapidly growing stem-cell and gene-therapy cluster is having a significant impact on California's economy.

A [2019 Economic Impact Report](#) highlights the impact of CIRM's programs, including:



CIRM's Economic Impact

\$10.7 billion in additional sales revenue

\$641 million more in state and local taxes

56,500+ new jobs created

The study, conducted by researchers at the USC Sol Price School of Public Policy, found about half of the new jobs were in medical and health-related research, manufacturing, and service sectors. New jobs were also created in sectors that indirectly support stem cell research, including construction and food services, for example. As the report's authors point out, "Because of the strong relationships of sectors in the California economy, all sectors in the state benefit from the existence of CIRM."

The regenerative medicine industry is poised for future growth, and as a leading global hub of stem cell and gene-based therapies, the state of California is expected to continue to be a global leader as the industry advances.

CIRM support leads to formation and growth of innovative biotech companies that attract private investment to the state.

A wide range of programs supported by CIRM have resulted in rapidly growing biotech companies that enrich the California economy—advancing science, supporting job growth, and generating tax revenues for the state:

CIRM Support Strategic Investments

Grantee	Focus area	CIRM support	Commercial milestone
Forty Seven Inc. <small>(via Stanford University)</small>	A new immunotherapy approach to target cancer	Early-stage research, preclinical development and clinical trials	Acquired by California-based Gilead Sciences, Inc. in April 2020 for nearly \$5 billion
jCyte	Adult stem cells to help battle a vision-destroying disease	Early-stage research, preclinical development and clinical trials	Raised \$252 million in licensing agreement
POSEIDA THERAPEUTICS	Cell-based immunotherapies for cancer	Preclinical development and clinical trials	\$283 million in venture capital financing, including \$75 million from Novartis
VIACYTE	Cell replacement therapy for type 1 diabetes	Early-stage research and clinical trials	\$115 million in venture capital financing and industry partnerships
TENAYA THERAPEUTICS	Gene therapies for heart disease	Early-stage research	\$142 million in venture capital financing

jCyte™ Seeing it through

Paul Bresge is no ordinary CEO, and the company he runs, jCyte, is no ordinary company. jCyte has developed a therapy to treat people with retinitis pigmentosa (RP), a rare vision-destroying disease.



Industry Partnership Funding

CIRM-funded clinical trials showed great promise and attracted offers from potential investors to the jCell therapy, but Bresge waited until he found a company he felt believed in the therapy as much as he did before bringing on a new investment partner. In May 2020, jCyte entered into a partnership with Santen Pharmaceutical in a deal worth up to \$252 million. The funding gives jCyte support to advance the clinical development needed to make jCell therapy more widely available.

Bresge says none of this would have been possible without CIRM and the guidance and advice of CIRM's Vice President for Therapeutics, Dr. Abba Creasey.

“CIRM supported our early preclinical data all the way through our late-stage clinical trials. This critical funding gave us the unique ability and flexibility to put patients first in each and every decision that we made along the way.”

Paul Bresge, CEO, jCyte, Inc.



Co-Funding/Partnership Funding/Additional Funding

To build something strong and lasting, you need a lot of help; no one person or company can do it all by themselves, not even CIRM. We can lay the foundations, but to get the most promising therapies to the levels that will help all the patients who would benefit from them, we need partners. Industry partners, in particular, help create access to additional capital and expertise.

When CIRM talks about industry funding, we mean investments into regenerative medicine projects that CIRM has previously or currently supports.

When investors see how the work we are supporting is not just showing promise, but also producing results, they are eager to be part of the program.

Since 2015, we have been able to attract \$8.9 billion in industry investment in researchers and companies we have supported. That money will help those promising therapies complete the clinical trials needed to show that they are both safe and effective. It will help them get approval from the Food and Drug Administration, so they are available to millions of people, not just the lucky few taking part in a clinical trial.

CIRM is a lender of first resort. We support projects long before most investors are ready to come on board. Our support, because we go to such great lengths to ensure that we only back the best science, helps take some of the risk out of a project, giving private funders more confidence that an early investment is a wise one.

New private funding has increased dramatically each year—from \$45.5 million in 2015 to more than \$5 billion in 2020.

New Industry Funding Investment by Year

2015	\$45.5 million
2016	\$153 million
2017	\$389 million
2018	\$1.3 billion
2019	\$1.5 billion
2020	\$5.5 billion

Total industry funding, 2015–2020:
\$8.9 billion

CIRM and Private Industry Partnership Impact

By putting early support behind promising research programs, CIRM has succeeded in bringing some of the best science to California, which has attracted significant additional scientific and commercial investments to the state and helped launch new cell-based therapy companies.

>1,000

Projects Funded

>\$8 billion

Industry Investment
Attracted

>40

Spin-Outs
Catalyzed

Helping patients defeat cancer

by activating their own immune systems

White blood cells keep our immune systems strong. Some of them have the job of attacking threats by surrounding and digesting (often described as “eating”) them and alerting other cells to come join the fight. But clever cancer cells can commandeer a “don’t eat me” signal, called CD47, preventing the white cells from doing their job.

Creating an antibody that blocks the CD47 signals and simultaneously boost “eat me” signals is the core focus of Forty Seven Inc.’s research to enable the patient’s own immune system to attack and destroy the cancer. This antibody is currently being evaluated in multiple clinical studies in patients with myelodysplastic syndrome, acute myeloid leukemia, non-Hodgkin lymphoma, ovarian cancer and colorectal carcinoma.

By investing in early innovative, and potentially transformative, research CIRM de-risks groundbreaking research. Armed with CIRM’s seal of approval and access to CIRM’s expert advisors and resources, research programs such as the anti-cancer project at Stanford University are well positioned to achieve early results and attract investors and commercial partners.

This groundbreaking anti-cancer discovery was made by researchers including Mark Chao at Stanford University in 2010. Other possible funding sources overlooked this promising science, but CIRM believed this discovery had potential and would one day empower patients to fight cancer with their own immune cells. CIRM not only provided early funding, but also assembled a Clinical Advisory Panel of experts to help advance the program and overcome hurdles as the research progressed from discovery through development and into clinical trials.



“CIRM’s support has been instrumental to our ability to rapidly progress Forty Seven’s CD47 antibody targeting approach.”

Mark Chao, M.D., Ph.D., co-founder and senior vice president of clinical development, Forty Seven Inc.

Fast-forward to April 2020:

the science discovered at Stanford (that later evolved into Forty Seven Inc.) was acquired by Gilead Sciences Inc. for nearly \$5 billion and combines Forty Seven’s expertise with Gilead’s strength in developing treatments that modify the immune system.

Developing California's workforce

Ushering in a new era of medicine needs much more than simply funding good science, it requires a talent pool prepared to support the rapid growth in regenerative medicine. That's why CIRM is developing a diverse community of future scientists through programs designed to introduce young people (and even career-changers) to regenerative medicine and supporting their academic and career pursuits in the field.

Sparking Interest: High School Programming

CIRM's education program begins with students in high school, where CIRM has made possible the curriculum for teaching regenerative medicine in California schools. In addition, the Summer Program to Accelerate Regenerative medicine Knowledge (SPARK) gives high school students a taste of what life in a stem cell research lab is like. SPARK summer internships expose students to world-class science and give them real-world context through community and patient outreach.

SPARK students reflect the diversity of California, including those who might otherwise not be able to afford a program like this. The majority of SPARK students are pursuing higher education in California as well.

Bridging from School to Career: Ongoing Education and Training

CIRM continues to nurture high-potential students through college, graduate school, and certificate programs, providing ongoing support to prepare candidates for jobs in California's university research centers and biotech companies.

Addressing unacceptable inequality in education and job skills is a top priority. By funding paid internships that provide critical experience, career connections, and a paycheck, CIRM's Bridges to Stem Cell Research Awards Program is leveling the playing field among all students and creating opportunities for students for whom a typical unpaid work experience might cause an economic hardship.

The Bridges program is integrated into the curriculum of 16 colleges and universities throughout California.

CIRM Bridges Program

By the numbers

1,400 participants

48% first-generation
college students

60% now employed in
research and development
positions

30% continue to further
advance their postgraduate
education



HELPING POINT THE WAY FORWARD

“Bridges gave me the tools and networks to be successful in the biotech industry. Being exposed to the potential of stem cells made me want to help harness that power and change the world.”

Sneha Santosh

Former Bridges Student

Sneha Santosh first heard about CIRM’s Bridges to Stem Cell Therapy and Research internship when she was graduating from the University of California, Davis, with a degree in microbial biotechnology and thinking about getting a master’s degree in biotechnology. She said the opportunity to be part of a program that is training the next generation of scientists was a game changer for her.

Today, she is a cell culture associate with Novo Nordisk, a leading global healthcare company in Fremont, California.

Through Bridges, she learned about stem cells’ power to treat a disease’s root cause rather than just the symptoms. She saw how these transformative therapies changed people’s lives. “Being part of the biotechnology industry was a way to be closer to that change,” she says. “I go to work every day knowing I am working toward the cure for type 1 diabetes.”

Sneha credits the Bridges program for helping her decide to pursue a career in biotech. In fact, it was pivotal.

A catalyst for scientific collaboration

Creating California Connections

Scientific collaboration between and among researchers, scientists, and private industry helps generate novel ideas and see them through. With one of the most skilled and highly educated regenerative medicine workforces right here in California, CIRM serves as both a matchmaker for connecting ideas to human and financial resources and a knowledge hub of stem cell and gene therapy information and expertise to further advance the science.

CIRM’s ongoing outreach and engagement with stem cell scientists throughout California creates connections that help the state’s public and private organizations find and hire “best and brightest” talent that is already in the state. Also, to attract top stem-cell talent (as well as private industry collaborators) to the state, CIRM maintains a presence in other markets where stem cell research is clustered and at world events that address and promote stem cell science.



2019 Stanford Drug Discovery Symposium

Maria Millan, President and CEO of CIRM, was invited to participate in this academic seminar on drug discovery. The panel addressed advances in research and technology for developing and testing novel diagnostics and therapeutics.

“I am honored to be a voice to a community that longs to be heard. I am committed to represent all Californians, whose tax dollars make potentially life-saving research possible, so they too feel well served.”

Ysabel Duron
Patient Advocate



REFLECT

*All her life, she has fought.
Against discrimination. Against social injustice. Against inequality.
Against cancer.*

For more than four decades, Ysabel Duron has been a pioneering (and award-winning) San Francisco Bay Area journalist — starting her career at a time when there were few Latinx individuals in TV news. Among the stories she covered was her own battle against Hodgkin’s lymphoma, a blood cancer. Her personal experience with cancer inspired the second act in her career: a health advocate for the Latinx community.

Ysabel founded Latinas Contra Cancer to provide education, screening access, and quality care to the most vulnerable in the Latinx community in California and the Latino Cancer Institute, a national network of cancer service providers that drives best practices, programs, and policies to eliminate the Latinx cancer burden. And now, she’s bringing her activist spirit, passion, and commitment to health equality to the CIRM Board.

Stem Cell Connection

science

and society

Symposia

CIRM hosts the Alpha Stem Cell Clinics Network Symposium, a public event held at a different location in California every year. Bringing together ideas, insights and varying points of view from experts, patients, and the public at large, these conferences provide a broad overview of current and future developments in the pursuit of FDA-approved stem cell-based therapeutics.

The 2019 symposium highlighted efforts of CIRM and others to develop a cure for sickle cell disease, a significant and costly health concern in California. (Learn more about the Cure Sickle Cell Initiative on page 22.)

Public Forums/Social Media

Public engagement and a better understanding of stem cell therapies is important for both science and society. CIRM engages the public in stem cell science through a wide range of public forums in communities throughout the state—ranging from high school presentations and TEDx talks, to live events hosted on social media, including “Ask the Stem Cell Team” on Facebook Live.



TEDx Gunn High School

CIRM's President and CEO, Maria Millan, delivered a talk on 21st century medicine at a TEDx event at the Henry M. Gunn Senior High School in Palo Alto, California. The presentation provided an easy-to-understand overview of stem cell and gene therapies.

Fighting unregulated clinics

As experts in the field of stem cell and gene therapy, it's also CIRM's duty to the people of California to effectively communicate the risks of untested, unregulated stem cell treatments that are not proven to be effective, are not approved by the FDA, and—in some cases—have even caused harm.

Stem cell therapies, like all new medicines, require diligent research, clinical trials, and regulatory approvals. CIRM is steadfast in its commitment to the advancement of legitimate stem cell research and fighting predatory purveyors of unproven and risky treatments.

Patient advocates

Patient advocates help bring perspective to CIRM's work so we can better serve Californian's unmet medical needs. These advocates led the charge that passed legislation that created CIRM in 2004 and have remained engaged with us every day since.

Unique to CIRM's approach, patient advocates are involved in all aspects of CIRM-supported research and development, including serving alongside scientists, researchers, and other experts on advisory panels that guide all recipients of CIRM funding as they progress through the translational and clinical stages of development.

“What CIRM really does is focus on patients and gives their voice and perspective to researchers and scientists who can get them the safe, reliable therapies that they need.”

Sneha Santosh

Researcher at Novo Nordisk, former Bridges student, and patient advocate

Responding to California's unmet medical needs

CIRM supports research that may one day provide new treatments for diabetes, cancer, and heart disease, as well as more than 70 other diseases, conditions, and injuries that affect millions of Californians. Research into rare and orphan diseases is also important in the advancement of medical science, since it can establish proof of concept, a starting point for therapies for other diseases and conditions.

In the U.S., health care costs for just six of the most chronic health conditions is about \$1 trillion annually; in California the cost is nearly \$100 billion each year. Therapies to reduce the severity or incidence of these chronic conditions have the potential to improve the health of millions of Californians and reduce health care costs for individuals, insurers, and the state.

Percent of total grants by disease/condition

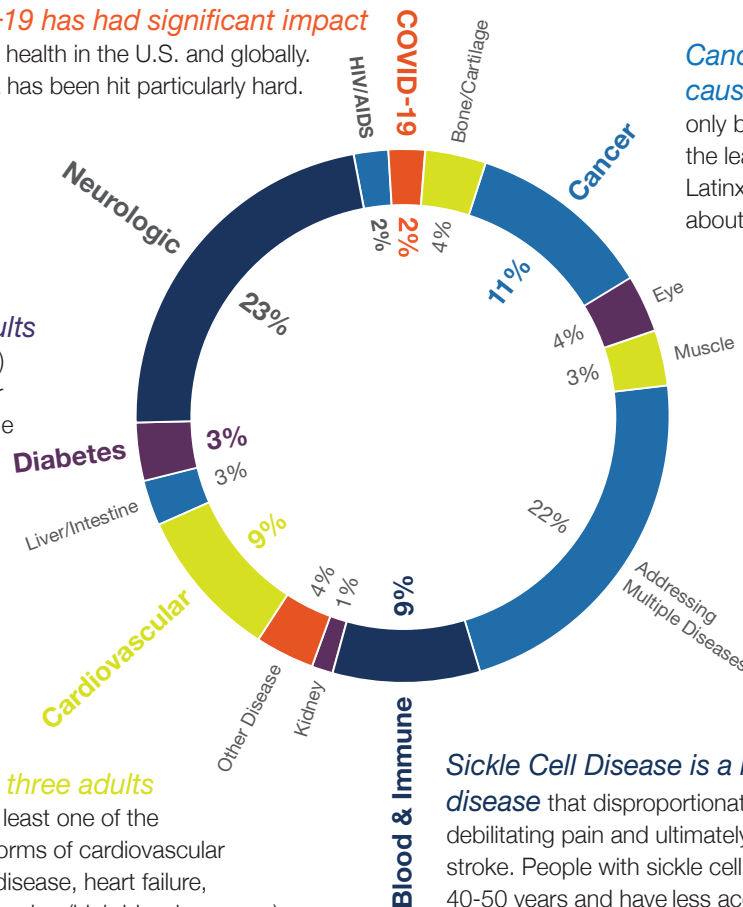
COVID-19 has had significant impact on public health in the U.S. and globally. California has been hit particularly hard.

Cancer is the second-leading cause of death in California, exceeded only by heart disease. Cancer is also the leading cause of death among the Latinx community, who represent about 40% of the population.

The majority of California adults (15.5 million people) have prediabetes or diabetes. Among the states, California has the greatest population with diabetes.

Nearly one in three adults are living with at least one of the most common forms of cardiovascular disease—heart disease, heart failure, stroke or hypertension (high blood pressure).

Sickle Cell Disease is a life-threatening, life-shortening disease that disproportionately affects African Americans that causes debilitating pain and ultimately leads to kidney failure, heart attacks and stroke. People with sickle cell disease have shorter life expectancy of 40-50 years and have less access to comprehensive medical care than people with other genetic diseases. CIRM in partnership with the National Heart, Lung, and Blood Institute is funding cell and gene therapy approaches to cure sickle cell disease.





VIACYTE

“With CIRM’s help, ViaCyte was able to make significant clinical progress. We continue to accelerate our mission to deliver transformative therapies for insulin-requiring diabetes.”

Paul Laikind
President and CEO, ViaCyte

Photo courtesy of Jamie Scott Lytle Photography

 CIRM-Funded **Clinical Trial**

Imagine a pod

the length and thickness of a business card, but only half as wide. Inside this pod are beta cells (derived from stem cells) that, when implanted under the skin, will be able to sense the level of blood sugar. If it’s too low, beta cells are designed to secrete insulin to restore blood sugar to a healthy level.

You don’t have to imagine; it’s real.

A San Diego company called ViaCyte developed it, with CIRM support, and is now testing it in people with type 1 diabetes.

Already, this cell-filled pod, called PEC-Direct, has proved it is durable and can survive in the body. More important, preliminary clinical trial data have shown that a subset of type 1 diabetes patients who had PEC-Direct implanted as part of a clinical trial were able to produce human C-peptide (a biomarker for insulin production)—something they hadn’t been able to do before.

ViaCyte’s President and CEO, Dr. Paul Laikind, says he believes this is just the start.

Creating Access: California-based clinical trials



40+ Disease Indications

130+ Clinical Trials

750+ Patients Treated

\$57M+ In Contracts with Commercial Sponsors

The CIRM-funded Alpha Stem Cell Clinics are a network of top California medical centers, given the name “Alpha” because they are the first of their kind, bringing the research, testing, and regulatory sides of clinical trials together under one roof to streamline and accelerate the process of getting stem cell therapies to patients.

Currently, more than 70 trials are being conducted through CIRM’s five Alpha clinics in 40 disease areas, including cancer, spinal cord injury, diabetes, heart disease, HIV, and diseases of blindness.

The Alpha Stem Cell Clinics Network provides statewide access to FDA-sanctioned clinical trials of therapies funded by CIRM (and others) that are sponsored by both academia and industry.

Tackling Sickle Cell Disease: For California and the world

Around 100,000 Americans, most of them African Americans, suffer from sickle cell disease (SCD). It’s a genetic condition that causes normally smooth blood cells to take on a rigid, sickle cell shape that blocks arteries, causes intense pain, damages organs, and shortens life expectancy. Current treatments can relieve pain and help prevent complications associated with SCD, but there is no cure for most people.

CIRM has entered into a unique partnership with the National Heart, Lung, and Blood Institute to develop a cure for SCD within five to 10 years, setting aside tens of millions of dollars to support the program. CIRM is currently funding cutting-edge research, including three clinical trials, aimed at developing novel treatments.

In addition, CIRM’s statewide Alpha Stem Cell Clinics Network has been named as a core program of the American Society of Hematology Sickle Cell Disease Collaborative Trials Network.

“We hear a lot about the moonshot for curing cancer, but a moonshot for curing sickle cell disease should also be possible. Sickle cell disease was the first genetic disease that was discovered, and wouldn’t it be great if it is also one of the first ones we can cure in everyone?”

Dr. Mark Walters, Director, Blood and Marrow Transplant, UC San Francisco Benioff Children’s Hospital Oakland



“I’m willing to do whatever it takes to help other kids. Somebody has to do it. I don’t have the money to donate to scientific conferences and stuff like that, but I can do this trial.”

Jordan Janz

Patient, pictured with researcher Stephanie Cherqui, Ph.D.

Looking at Jordan Janz today, you would not know he was battling a rare disease. For years, he had daily injections and took 56 pills just to keep his condition under control, because there was no cure. He has cystinosis, a genetic mutation that, over time, causes damage to every cell and organ in the body. A short life was expected.

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PIONEER

Today, Jordan is a pioneer in the fight against cystinosis. In 2019, he became the first patient to try a unique gene therapy as part of a CIRM-funded clinical trial through the University of California, San Diego (UCSD) School of Medicine, a member of CIRM’s Alpha Stem Cell Clinics Network.

The therapy, developed after more than a decade of research by Stephanie Cherqui, Ph.D., and her team at UCSD, takes a patient’s own blood stem cells and, in the lab, inserts a functional version of the cystinosis gene, replacing the mutated gene. The corrected cells were returned to Jordan, where they are intended to reduce the crystal buildup that has been damaging his organs. He knows there are no guarantees. Even so, he’s glad to be a pioneer in the fight against this debilitating disease.

Financial Review: 2019-2020 18-Month Accounting

Financial Reconciliation, June 30, 2020

	January 1, 2019	June 30, 2020
Committed Balance	\$2.6 billion	\$2.7 billion
Uncommitted Balance	\$144 million	\$45 million
Balance Under Active Management	\$351 million	\$211 million
Number of Programs Under Active Management	214	181

2020-2021 Approved Budget As of June 30, 2020

Program	Allocation
Clinical – Sickle Cell Disease	\$28 million
Clinical	\$17 million
COVID-19	\$5 million
Discovery	\$2 million
Discovery/Progression	\$2 million
Grantee Meeting	\$250,000
Total	\$54 million

All numbers rounded to the nearest million.

2019 CIRM Game Ball Winners



People Behind the Performance

CIRM's greatest resource is its people. Every day, they show up, committed to bringing their expertise to bear to further our mission and speed up therapies to patients with unmet medical needs.

The team is relentless in its desire to help others, resourceful in finding ways to do more with less,

and works closely together to achieve the agency's goals. It is not unusual for members of CIRM's team to go above and beyond the call of duty to help others. To them, we award a Game Ball, to honor their role in making us all a better and more successful team.



Kent Fitzgerald
Director of Discovery and Translation



Maria Bonneville
Vice President of Administration, Executive Director to the CIRM Governing Board



Yimy Villa
Marketing Communications Manager



Lauren Rath
Grants Management Specialist (former)



Gil Sambrano
Vice President, Portfolio Development and Review



Gabe Thompson
Director, Grants and Operations (former)



Koley Lambright
Administrative Assistant, Therapeutics



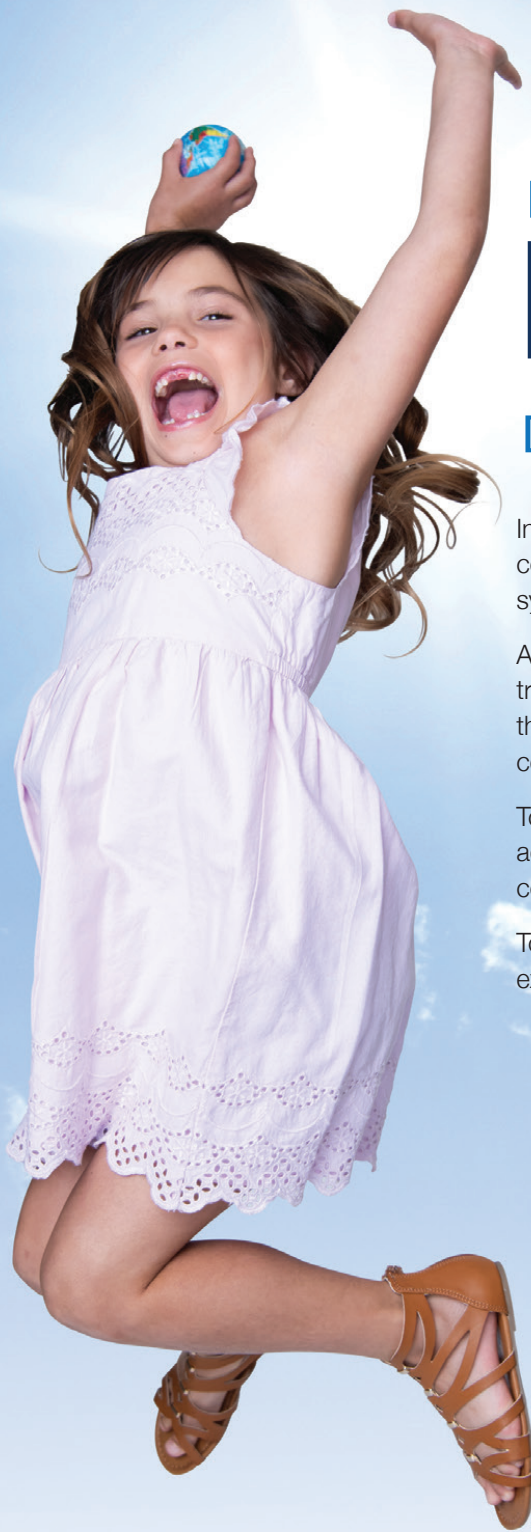
Ben Huang
Acting General Counsel



Bill Gimbel
Director, Information Technology



Denise D'Angel
Associate Director, Human Resources



Learning. Growing. Living.
Right now.

Defining Perseverance

In Southern California in 2012, Evie was born with severe combined immunodeficiency. She had no functioning immune system, and this left her vulnerable to infections that could kill her.

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

Today, thanks to the perseverance of dedicated researchers, advocates, and medical professionals—and CIRM's unrelenting commitment—Evie is cured.

Tomorrow, she will continue to grow, learn, and enjoy exploring and living life's adventures with her family.