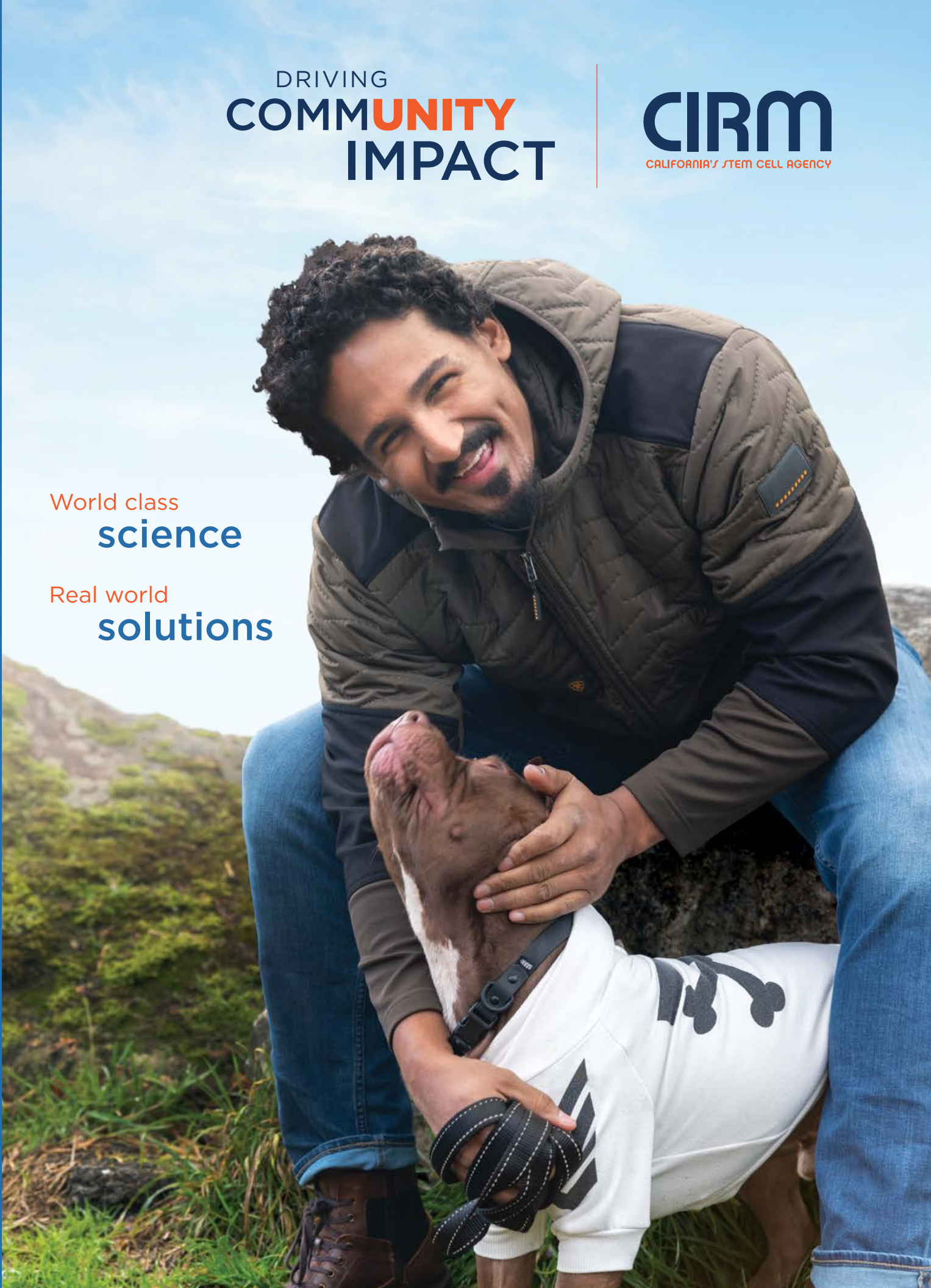


DRIVING
COMMUNITY
IMPACT

CIRM
CALIFORNIA'S STEM CELL AGENCY

World class
science

Real world
solutions



MISSION

Accelerating world-class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world.

The people of California created The California Institute for Regenerative Medicine (CIRM) when they approved Proposition 71 in 2004. The passage of Proposition 14 in 2020 renewed our funding and assured our work can continue. We honor the trust the people of California have placed in us and strive to fulfill our mission.

We partner with patient advocates, communities, academia and industry to bring the most promising regenerative medicine to the people of California and the world.

With \$5.5 billion in funding and more than 237 active stem cell and gene therapy programs, CIRM is one of the world's largest institutions dedicated to bringing stem cell and gene therapies to the patients who need them.

➤ Evie, a participant in a CIRM-funded clinical trial is shown on the cover with his dog and here with his partner Danielle.

All photos of Evie courtesy Jaquell Chandler

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COMMUNITY IMPACT

At CIRM, we know it is important to honor, support and positively impact the many communities we serve. We hope this report demonstrates our continued commitment to advancing science that brings new treatments and cures for patients with unmet medical needs.

Ushering 21st century cell and gene therapies into clinical practice and delivering them to patients involves bringing together many different communities, such as researchers, doctors and nurses, patients, patient advocates, industry partners and many more. By uniting these communities, and fostering information sharing, collaboration and diverse insights, we will advance regenerative medicine from scientific research to patient recovery.

That's community impact.

Executive Letters

Dear Fellow Californians,



It seems only appropriate to begin this letter with a heartfelt thanks to you, the people of California, for passing Proposition 14 in November 2020 and allowing us to continue building on what we began in 2004.

We have come a long way in the last 18 years. CIRM has directly funded more than 80 clinical trials and provided critical early support to more than two dozen other projects that resulted in clinical trials. We have funded and supported a pipeline of medical research from initial scientific discovery to development and testing. At the same time, we are training a new generation of regenerative medicine scientists.

Despite our strong progress, we recognize that we have much more work to do. We are driven to find cutting-edge therapies and ensure equitable access to them. Treatments from CIRM-funded research must be available and affordable to all the people of California. Our new Accessibility and Affordability Working Group and new Patient Assistance Program are dedicated to this critical challenge.

California has always been a world leader. We know this investment will impact Californians and will help provide treatments for conditions that affect millions of people around the world. Our work is local, our impact is global.

With kind regards,

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

Dear Friends,

The people of CIRM are motivated by our mission: accelerating world class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world. We have crafted a thoughtful and ambitious Strategic Plan to guide our success.

Three areas of focus frame our efforts for the next five years to advance world class science, deliver real-world solutions and provide opportunity for all Californians. To achieve these goals, we have expanded our team and are positioning CIRM to meet new challenges and emerging opportunities.

We are actively taking on the important goals of incorporating diversity, equity and inclusion in all our programs. This report presents exciting steps toward building bridges with communities and partners,



including scientists, healthcare professionals, cell and gene therapy manufacturers, and other industry groups.

Our clinical programs are making great progress and our Alpha Clinics continue to deliver results. This report shares the genuine impact CIRM is having on medical science and on the lives of many individuals. Their stories motivate and give us fresh purpose every day.

In closing, we are honored to continue demonstrating California's innovation and leadership that will have a lasting positive benefit for the communities we serve.

Sincerely,

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



Advance
World Class Science

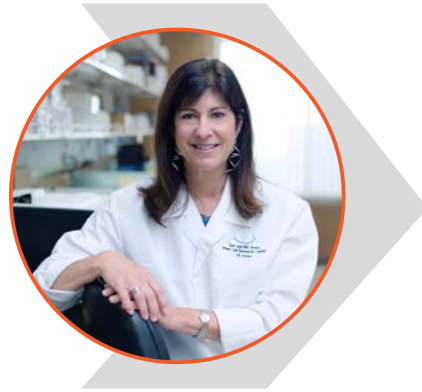
Deliver
Real World Solutions

Provide
Opportunity for All

CIRM STRATEGIC PRINCIPLES

CIRM is energized to achieve our mission. We've prepared for success by developing a specific, measurable and achievable strategic plan that will serve as our roadmap for the next five years.

Our strategic plan is built on three guiding principles: advance world class science, deliver real-world solutions and provide opportunities for Californians. The decision to make these principles the core of our plan is the result of thoughtful discussion with stakeholders throughout California and across scientific research and drug development communities.



Advancing World Class Science

CIRM will leverage collective scientific knowledge to inspire collaborative research that addresses Californians' unmet medical needs.

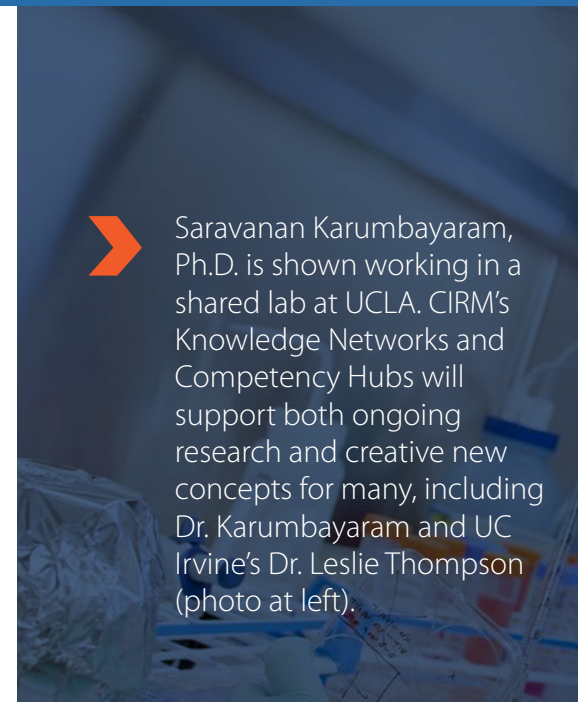
The regenerative medicine field is advancing rapidly, but the tendency of biomedical research to happen in silos—with few structured opportunities to share knowledge, expertise and data—is hindering even faster progress. Collaborative science is essential. We are actively developing programs that promote data sharing and resource sharing, as well as including diverse insights and perspectives.

Addressing Neurological Disorders

Since the passage of Proposition 14 in November 2020, we have invested more than \$88 million in 23 projects for conditions and disorders affecting the central nervous system (CNS), which includes the brain, eyes and spinal cord. This investment in CNS represents 32.5% of CIRM's research and development funding spanning Discovery, Translational and Clinical programs.

We are committed to investing at least \$1.5 billion—more than double what we funded between 2006 and 2020—in treatments that target CNS conditions, such as epilepsy, multiple sclerosis, Parkinson's disease and Alzheimer's disease, as well as stroke, migraine and other brain disorders.

When CIRM-supported Neurona Therapeutics treated the first patient in its clinical trial for a drug-resistant form of epilepsy, it marked a significant milestone for the company, culminating 20 years of research. For CIRM, it highlighted the impact of our \$14 million investment spanning Discovery, Translational, and Clinical stage research programs, highlighting our commitment to taking the most promising science from the bench to the bedside.



Saravanan Karumbayaram, Ph.D. is shown working in a shared lab at UCLA. CIRM's Knowledge Networks and Competency Hubs will support both ongoing research and creative new concepts for many, including Dr. Karumbayaram and UC Irvine's Dr. Leslie Thompson (photo at left).



Photo courtesy UCLA

Creating a Culture of Collaboration

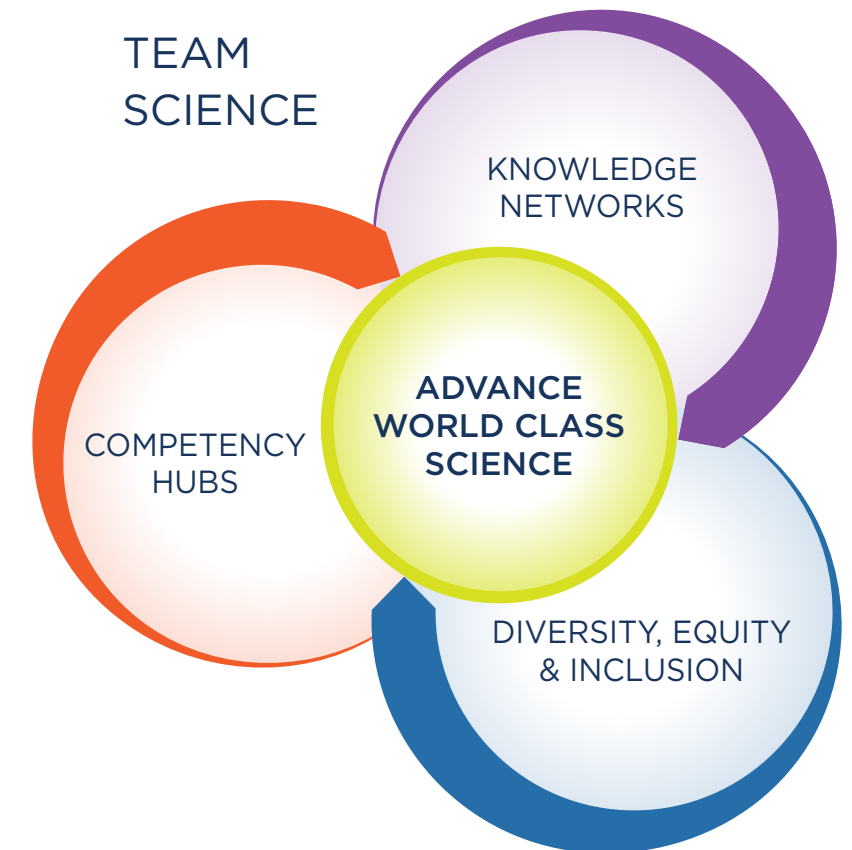
CIRM is advancing two key initiatives—Knowledge Networks and Competency Hubs—to build a broad culture of collaboration to propel research forward and impact human health.

Knowledge Networks

Too often, research is done in isolation and lessons learned by one scientist are not shared with others. Knowledge Networks will help ensure that information and data generated by CIRM-funded research projects will be shared with other scientists and researchers.

Competency Hubs

Competency Hubs are collaborative environments that encourage the sharing of resources, expertise and training. Because they enable researchers to work together with a team-science approach, Competency Hubs will accelerate discovery and therapy development.



Unlocking the Secrets of the Tooth Fairy

← Alysson Muotri, Ph.D.
CIRM-funded researcher

Photo: Courtesy UC San Diego

➤ Dr. Muotri uses stem cells to create organoids—structures that mimic the brain. Pictured right and below left, culturing brain organoids. Below center and right, images of induced pluripotent stem cells that have been pushed to form organoids (center) or plated to differentiate into individual neurons (right).

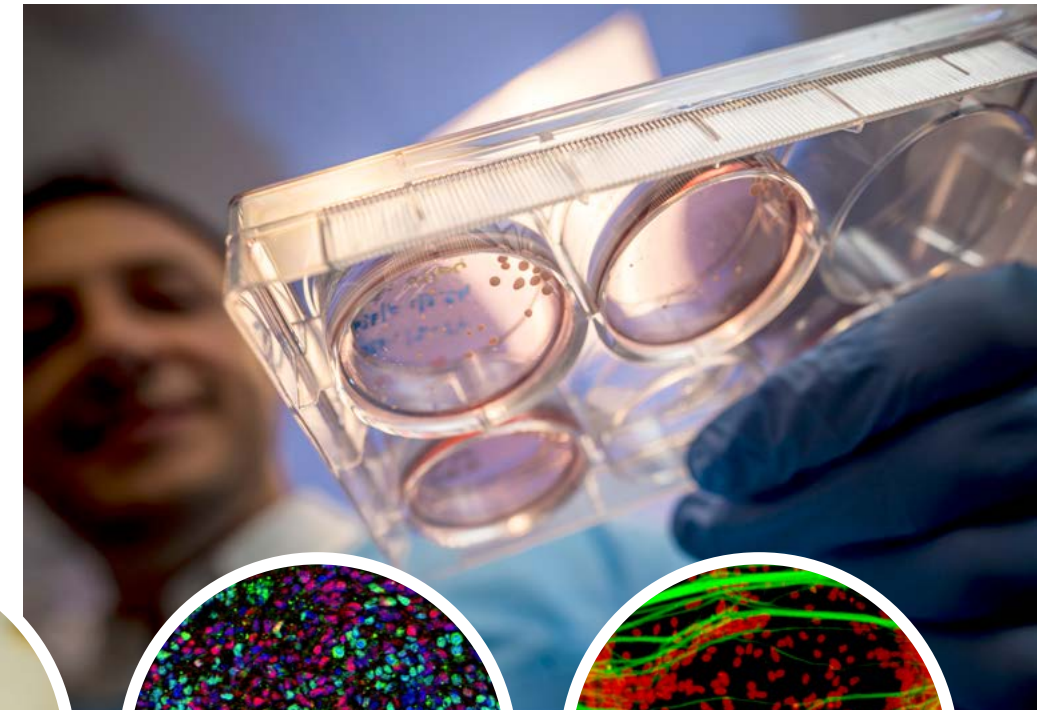
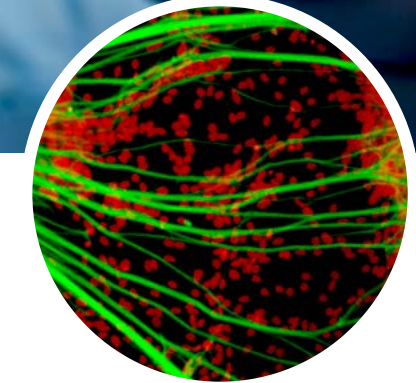
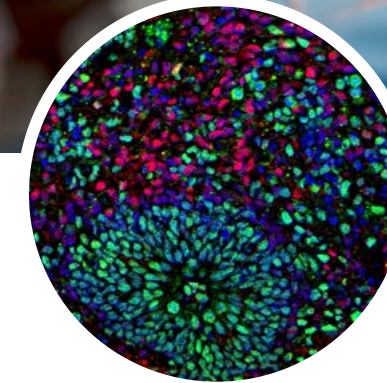


Photo: Erik Jepsen, UC San Diego

For Alysson Muotri, Ph.D., professor of pediatrics and cellular molecular medicine at the University of California, San Diego, trying to unlock the secrets of the brain isn't just a matter of scientific curiosity; it's personal. He has a son with autism spectrum disorder (ASD), which is caused by differences in the brain. Dr. Muotri is committed to finding ways to help his son, and millions of others like him, reduce the symptoms that interfere with their daily activities and quality of life.

Dr. Muotri created the Tooth Fairy project. Parents donated baby teeth from children with ASD and he took cells from those teeth and turned them into the kind of brain cells affected by autism. Dr. Muotri is using those cells to identify characteristics of the brain that may be linked to autism.

Dr. Muotri also is using stem cells to create brain structures that mimic certain actions and activities of the brain. He uses these to gain deeper knowledge of how the brain works and to evaluate drug safety before human testing.

He says he still gets excited seeing how these cells work. "It's amazing, it's a miracle. Every time I see it, it's like seeing dolphins in the sea because it's so beautiful!"



Delivering Real World Solutions

CIRM is overcoming critical bottlenecks to accelerate approval of therapies for all patients.

The journey from early discovery to clinical trials to commercially available medical treatments is lengthy, costly and fraught with obstacles. CIRM is addressing these hurdles while actively engaging with all Californians, including communities that have been overlooked in the past. To do this we are expanding our Alpha Clinics Network, creating Community Centers of Excellence, and addressing bottlenecks in manufacturing to enable us to bring these therapies to the public.

➤ Ja'Ceon (shown above in circle) was born with the rare and often fatal immune disorder Severe Combined Immunodeficiency (SCID). In 2017, he participated in a CIRM-funded clinical trial through UC San Francisco that corrected the genetic mutation that causes SCID. He now has a new immune system and is enjoying life. Evangelina (right) participated in a similar SCID clinical trial in 2012 at UCLA. Today she is an adventurous and healthy 10-year-old.

Expanding Basic Biology Research

Discovery, or basic biologic research, is the stage where researchers develop and pursue creative, inventive and innovative concepts.

The opportunity for groundbreaking medical discovery at the earliest stage is why CIRM created the DISC-0 Foundation Awards. The awards support basic research that explores gaps in our knowledge about disease and how best to tap into the potential of stem cells and genomics.



City of Hope

UC Irvine/
UC Los Angeles

UC
San Diego

UC
Davis

UC
San Francisco

Future Sites
Coming



CIRM
ALPHA CLINICS
Delivering Patient Treatments

Photo courtesy City of Hope



➤ Translating years of research into real-world patient treatments is the work of CIRM Alpha Clinics. Shown here, staff from the City of Hope Alpha Clinic.

1,000+
PARTICIPANTS

200+
CLINICAL TRIALS

40
DISEASES

\$80
MILLION
INVESTMENT

CIRM Alpha Clinics

Developing a stem cell treatment or gene therapy can take years, even decades, of scientific research. Delivering that therapy to a patient can be equally challenging, unless the patient is in a medical facility that has a staff skilled and experienced in delivering this care. The CIRM Alpha Clinics Network features five world-class medical facilities with a common goal of streamlining and accelerating the delivery of stem cell and gene therapies to people in need.

For example, CIRM's Alpha Clinics Network played a role in the City of Hope blood stem cell transplant that helped a man, diagnosed with HIV in 1988, go into long-term remission. This is only the fifth time that has ever happened.

To date the Alpha Clinics have carried out more than 200 clinical trials addressing 40 different diseases involving more than 1,000 participants. The Alpha Clinic model is proving so successful that the CIRM Board has approved investing an additional \$80 million to expand the network and enhance its ability to help patients around the state.

Partnering for Progress

Beyond funding important research, CIRM facilitates unique partnerships between academic researchers and medical technology and drug development companies to help spur advancement of potential cell- and gene-based patient treatments.



Connections between CIRM-funded research projects and the broader regenerative medicine industry help deepen the impact of CIRM funding on regenerative medicine research. CIRM-funded research projects have attracted more than \$23.4 billion in additional investment from industry partners.

Academic research projects funded by CIRM have created, or “spun out”, at least 50 startup companies, many of which are actively building the California regenerative medicine industry, providing jobs and contributing to the state’s economic vitality.

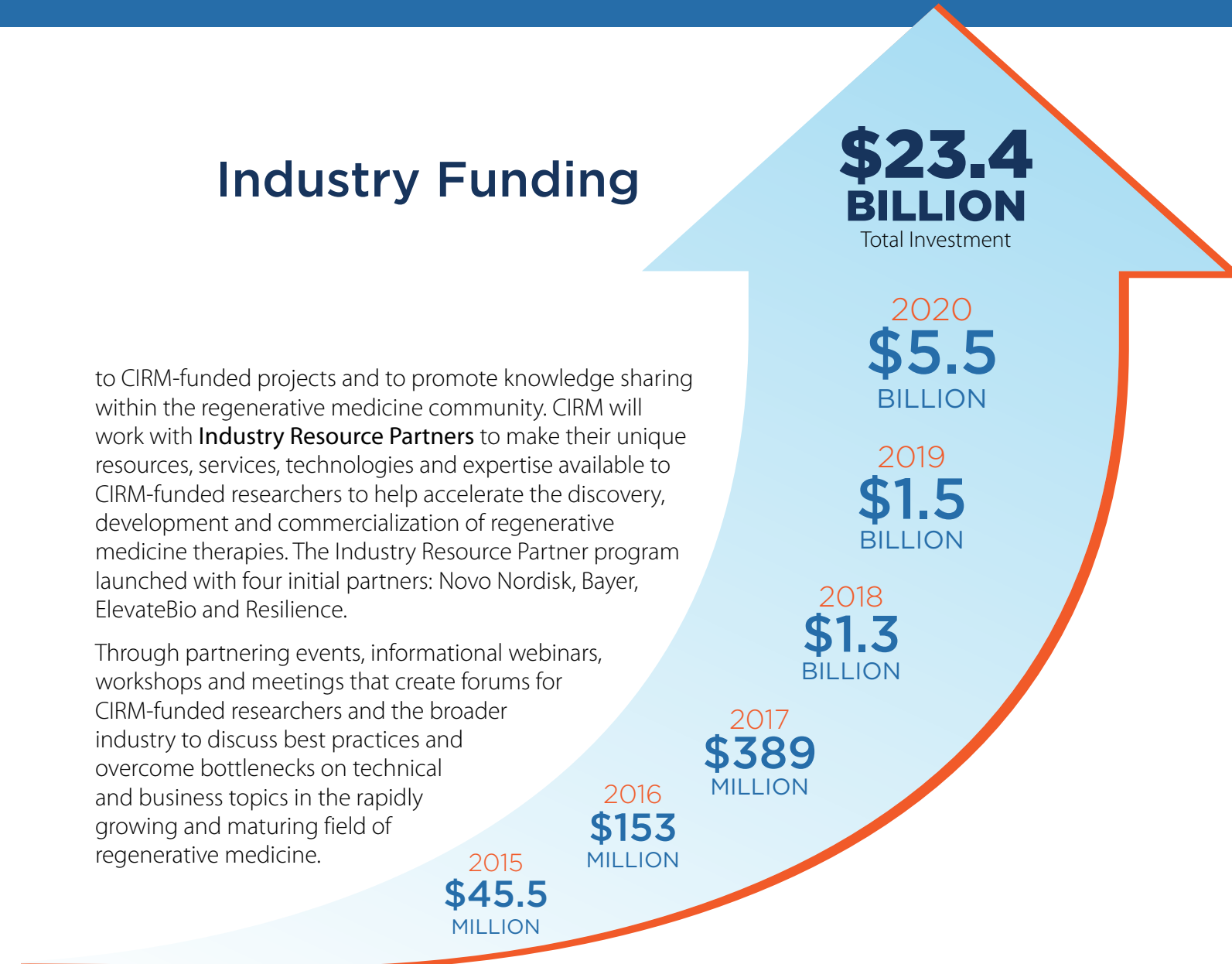
CIRM’s **Industry Alliance Program** connects biopharmaceutical companies and biotech investors to stem cell, gene and regenerative medicine research programs in our portfolio to help them advance through further development.

We recently expanded the Industry Alliance Program to help make industry resources more accessible

Industry Funding

to CIRM-funded projects and to promote knowledge sharing within the regenerative medicine community. CIRM will work with **Industry Resource Partners** to make their unique resources, services, technologies and expertise available to CIRM-funded researchers to help accelerate the discovery, development and commercialization of regenerative medicine therapies. The Industry Resource Partner program launched with four initial partners: Novo Nordisk, Bayer, ElevateBio and Resilience.

Through partnering events, informational webinars, workshops and meetings that create forums for CIRM-funded researchers and the broader industry to discuss best practices and overcome bottlenecks on technical and business topics in the rapidly growing and maturing field of regenerative medicine.



Industry Alliance Program Partners:

Spinout companies:



Building Regenerative Medicine Manufacturing Capacity

Developing treatments for conditions and diseases that lack a known cure will only help patients if the treatments are available to them. Producing—or manufacturing—these treatments at scale is a critical component of the regenerative medicine ecosystem.

An increase in the number of cell and gene therapies making their way through clinical trials means future demand for high-quality, rigorously tested treatments will also increase. And because each therapy is unique, great pressure falls on existing manufacturing facilities to meet the demand.

CIRM is building a network of regenerative medicine manufacturers in California with the ability and capacity to produce the consistent, safe and reliable cell and gene based products needed to treat patients here and around the world. This collaborative approach that links research, development and manufacturing increases the likelihood for therapies to get regulatory approval. The network also will help build, educate and train a diverse California workforce to do this vital work.

Gene Therapy

CIRM is partnering with the Foundation for the National Institutes of Health to join the Bespoke Gene Therapy Consortium (BGTC), a public-private partnership that brings together the National Institutes of Health, the U.S. Food and Drug Administration, and multiple public and private sector organizations to streamline the development and delivery of gene therapies for rare diseases. We believe the BGTC is a critically important partnership that can advance therapies, especially for rare diseases that currently lack effective treatment.



Treating Birth Defects Before Birth

When Michelle and Jeff learned they were expecting a baby, they were elated. Then an ultrasound exam at 20 weeks into the pregnancy showed the fetus had spina bifida, a birth defect that occurs when the spine and spinal cord don't form properly. Spina bifida can result in paralysis and other serious complications.

The couple were referred to a clinical trial at the University of California, Davis, being carried out by internationally renowned fetal and neonatal surgeon, Diana Farmer, M.D., and her colleague Aijun Wang, Ph.D. In this clinical trial, which built upon earlier CIRM-funded research, the spinal defect is repaired by applying stem cells from a donor

placenta, which are seeded onto a synthetic scaffold, and applied to the spinal cord defect while the baby is still in the womb.

Michelle and Jeff's son Toby was just the second patient to receive this treatment. Michelle said the surgery was challenging but the birth of her baby made it all worthwhile. "Holding him for the first time, it was like, I can't believe we did this. We made it. We went into this not knowing if this would even work."

At three months, Toby's progress looks promising. Jeff and Michelle know problems could emerge later but for now they are just grateful to have been part of this trial.



Baby Toby, who received a CIRM-funded stem cell treatment while still in the womb, and his parents Michelle and Jeff.

Tackling Sickle Cell Disease from Within

Evie (right) was born with sickle cell disease, a life-threatening and extremely painful condition that affects an estimated 100,000 Americans, most of them Black and Latino. Globally, about 25 million people carry the gene responsible for the condition.

People with sickle cell disease have blood cells that are brittle and shaped like a hook (or sickle) rather than smooth and round. Instead of flowing easily through the veins and arteries, the sickle shaped cells can create clogs causing intense pain, organ damage and stroke.

By age 18, Evie was having severe pain crises once or twice a month, often ending up in the hospital. His condition finished his high school football career and cost him a college scholarship. By his mid-20s Evie was constantly tired and he struggled to keep a job.

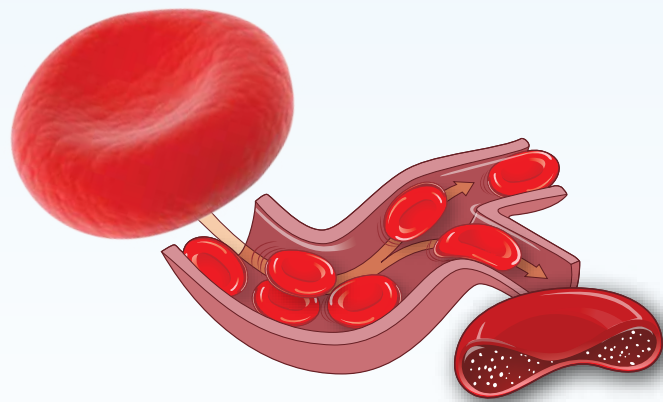
Then he took part in a CIRM-funded clinical trial. Don Kohn, M.D., researcher and professor at the University of California Los Angeles, and his team, collected Evie's blood-forming stem cells and, in the lab, inserted a gene that prevented the sickling of the cells. Those modified cells were returned to Evie to create a new blood supply. Two years later Evie says he hasn't had any pain crises requiring medications or trips to the emergency room.

CIRM has invested more than \$65 million in 12 potential new treatments for sickle cell disease including five clinical trials, and has a unique partnership with the National Heart, Lung and Blood Institute as part of their Cure Sickle Cell Initiative. The goal is to help all the Evies of this world have a chance to lead a full life.



Normal Blood Cell

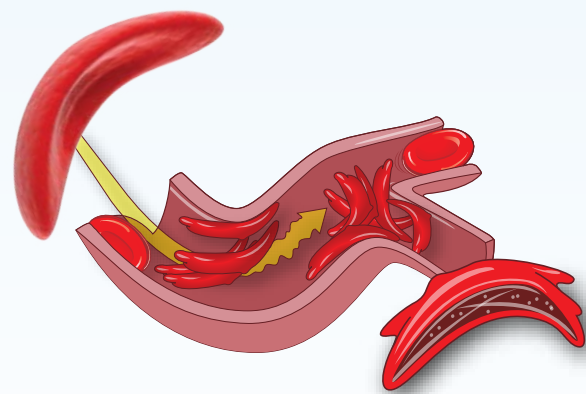
Healthy red blood cells, flow freely within blood vessels.



Rounded and pliable shape of healthy hemoglobin

Sickle Cell

Abnormal sickle cells block blood flow within blood vessels.



Rigid cell-form strands that cause a sickle shape



Providing Opportunity for All

CIRM is building inclusive participation opportunities for all stakeholders, from students to the workforce to patients.

The diverse cultures and communities that make California a great state can also make health care better for all people. By infusing diverse insights into health care—from research to patient care—CIRM is helping to ensure an inclusive experience for everyone.

Education and Training

CIRM offers an array of education and training programs to build a diverse workforce that will fuel California’s regenerative medicine industry and unite new treatments with the underserved Californians who need them. Training and educating the people of our state who come from varied backgrounds brings new perspectives and insights to the field of regenerative medicine.

From its start, CIRM has been committed to supporting the next generation of researchers, scientists and innovators. We have helped thousands of talented individuals—from high school and college students to postdoctoral and clinical fellows—gain hands-on laboratory experience working with world-class stem cell and gene therapy researchers right here in California.

CIRM’s newest program, COMPASS, identifies untapped talent within populations that have historically been under-represented in the scientific community, provides them culturally competent mentorship and helps them develop skills needed in California’s growing regenerative medicine field.



Photo courtesy Maziar Shah Mohammadi

CIRM is inspiring students of all ages and investing in future generations of researchers. Above, SPARK high school students are on a tour of UC Berkeley Cell Culture and Advanced Imaging Facilities. On facing page, Alessandra Rodriguez y Baena, a former Bridges student, is working toward her Ph.D. at UC Santa Cruz.

For Everyone. Everywhere.



EDUCATION PORTAL

SPARK
High School

BRIDGES & COMPASS
Undergrad and Masters

SCHOLARS
Graduate and Postgrad

COMMUNITY IMPACT

Accessibility and Affordability

To truly serve the diversity of California, cell and gene therapies must be accessible and affordable to all people in the state. A new Medical Affairs and Policy team at CIRM is working toward this goal with our new Accessibility and Affordability Working Group (AAWG).

The AAWG will identify ways to ensure everyone—particularly communities of color and low-income communities—has equitable access to needed care. Members of the AAWG bring deep experience and expertise to the group, including insights from the worlds of policy and patient advocacy, health insurance, medical care, the pharmaceutical industry and philanthropy. They are well versed in the challenges facing patients.

Through Proposition 14, a **Patient Assistance Fund** has been set up to cover the costs of treatments developed from CIRM-funded research. To date, the fund holds \$15.6 million. All future proceeds arising from CIRM-funded research will be added to the account.

Community Outreach

As a state agency, CIRM has a responsibility to keep the people of California informed about our investments, the scientific progress we are making and the economic benefits our work brings to the state. We also have a wider role to play in reaching out to communities that have often been overlooked in the past.

We strive to be a trusted source of information about scientifically backed stem cell and gene therapies, and to address the fears and mistrust some people may have about them.

That's why we have developed a new outreach plan. Our plan uses multiple avenues to reach as many people as we can, including: mainstream media, social channels, in-person talks and presentations through community organizations such as at Rotary clubs, connecting with patient advocates and participating in scientific conferences.

CIRM is working to meet people where they are. We partner with trusted individuals and organizations in these communities to help raise awareness about stem cell and gene therapies.

◀ CIRM participated in the 2022 San Francisco Pride Parade to build relationships and share the importance of regenerative medicine in treating HIV/AIDS and other diseases.



Opportunity of a lifetime: impacting the lives of others.

CIRM funds education and research training programs to give students the opportunity to explore stem cell science right here in California.

One such project, the Bridges to Stem Cell Research Program, helps train future generations of scientists by preparing undergraduate and master's degree students from California universities and community colleges for careers in stem cell and regenerative medicine research. By the end of 2022, nearly 1,800 Bridges trainees will have completed this CIRM-funded internship program.

As an undergraduate student majoring in biological sciences at California State University, Fullerton, Samira Alwahabi was one of the participants in last year's Bridges internship program. She completed her internship in the Calvin Kuo Lab at Stanford University. The experience, she says, was nothing short of incredible.

"I was able to be a part of cutting-edge stem cell research and I gained incredible mentors and friends within academic medicine who push me to be the best version of myself," Samira says.

After completing her internship last year, Samira graduated with a degree in cell and developmental biology. She is currently working in the Kuo Lab as a technician and will soon apply to medical school. Samira plans to become a physician and use her research experience to better understand medical innovations that improve patient care quality.

◀ Samira Alwahabi (left) and fellow CIRM Scholar Alan Napole graduated from CSU, Fullerton with honors. Both plan to be doctors.

The People Powering CIRM



When Proposition 14 passed in 2020, a long period of uncertainty about the future of regenerative medicine in California—and CIRM itself—ended. But even during that uncertain time, the team at CIRM remained committed to our mission.

When COVID forced us to work from home, the team stepped up to meet the new challenges of accelerating the research and development of regenerative medicine. Plus, we welcomed new, extraordinarily talented team members to help advance our work.

To those who stayed and those who joined more recently, we say a big thanks.

By the Numbers

80 CIRM Clinical Trials

Clinical trials are the final steps on the path to FDA approval of new therapies for patients.

40 Diseases/Conditions

Research projects to address a wide range of health issues, from arthritis to Alzheimer's disease, cancer, heart disease and stroke.

3,400 Participants in Clinical Trials

Pioneers who have signed up and consented to be part of a clinical trial CIRM is funding.

31 TAPs and CAPs

Translational and Clinical Advisory Panels that help CIRM-funded researchers accelerate development of their therapies and anticipate future challenges.

237 Active CIRM-Funded Projects

11 RMAT Designations

CIRM-funded projects recognized by the FDA as Regenerative Medicine Advanced Therapies designed to expedite FDA regenerative medicine review.

5 CIRM Alpha Clinics

CIRM-supported medical facilities with staff experienced in administering regenerative medicine therapies. Alpha Clinics have run more than 200 clinical trials.

\$23+ Billion in Industry Partnerships

Additional investments in CIRM-funded research from biotechnology and pharmaceutical companies and other industry partners.

50+ Spinouts

Number of startup companies with roots in CIRM-funded research projects.

3,385 Next Generation Scientists Trained

High school and college students, postdoctoral candidates and clinical fellows involved in CIRM-supported education and training programs.

ICOC: Guiding Our Mission

Creating a new state agency from scratch is no easy task. But that's what the CIRM Board, the Independent Citizens' Oversight Committee (ICOC), was charged with doing after the voters approved Proposition 71 in 2004. The Board helped develop the rules, regulations, procedures and policies that guide the work CIRM does, ensuring we live up to the highest ethical standards and never lose sight of advancing science and helping patients.

Proposition 14 added six new members to the ICOC, including nurses, mental health experts and professionals from parts of the state not represented on our Board in the past, adding important and diverse perspectives to CIRM.

ICOC Members

Jonathan Thomas, Ph.D., J.D.
 Senator Art Torres (Ret.), J.D.
 Haifaa Abdulhaq, M.D.
 Mohamed Abousalem
 Kim Barrett, Ph.D.
 Dan Bernal
 George Blumenthal, Ph.D.
 Linda Boxer, M.D., Ph.D.
 Carol Christ, Ph.D., M.Ph.
 Le Ondra Clark Harvey, Ph.D.
 Deborah Deas, M.D., M.P.H.

Anne-Marie Duliege, M.D.
 Ysabel Duron
 Mark Fischer Colbrie, M.B.A.
 Fred Fisher, MSW, LCSW
 Elena Flowers, Ph.D., R.N.
 Judith C. Gasson, Ph.D.
 Larry Goldstein, Ph.D.
 David Higgins, Ph.D.
 Stephen Juelsgaard, D.V.M., J.D.
 Rich Lajara
 Pat Levitt, Ph.D.
 Linda Malkas, Ph.D.

Shlomo Melmed, M.D.
 Christine Miaskowski, R.N., Ph.D., FAAN
 Lauren Miller Rogen
 Adriana Padilla, M.D.
 Joe Panetta
 Al Rowlett
 Marvin Southard
 Michael J. Stamos, M.D.
 Kristiina Vuori, M.D., Ph.D.
 Karol E. Watson, M.D., Ph.D., FACC
 Keith R. Yamamoto, Ph.D.

Strategic Investments

CIRM’s mission and strategy rely on five critical areas of investment: Infrastructure, Education, Discovery Research, Translational Research and Clinical Research. Each is an essential pillar that enables CIRM to advance stem cell and gene therapy research at every level.

Budget for Fiscal Year 2021-2022: \$474.3 million

Funds Approved*	\$ 206,481,451
Funds Remaining	\$ 4,534,615,942
Balance Under Active Management	\$ 428,086,226
Number of Active Project Awards	237

*Does not include allocated budget of \$162.2 million for pending awards for Alpha Clinics, COMPASS and DISC-0 programs

Approved Budget for Fiscal Year 2022-2023: \$426.7 million



Infrastructure

\$80 million

Our infrastructure program builds real and virtual centers that provide the resources, expertise and information needed to advance CIRM’s mission.



Education

\$1.3 million

Through our education programs, CIRM is helping train CIRM Scholars, the next generation of regenerative medicine scientists and technicians needed to advance the field.



Discovery

\$106 million

We invest in early-stage basic, or Discovery, research that advances our understanding of how stem cells and genes work, and explores new and groundbreaking stem cell and gene therapy treatments and technologies.



Translational

\$70.4 million

CIRM helps the best Discovery-level research advance to the next level by establishing, supporting and testing the foundational work required for clinical trial applications.



Clinical

\$169 million

We are building a world-class therapeutics portfolio and supporting each project with a Clinical Advisory Panel and other resources to increase its chances of success into and through clinical trials.

Strength Forged from Adversity

Regina Karchner feels like she’s always been an advocate for patients with brain cancer. When she was just three years old, her father died of a brain tumor. At 16, Regina was diagnosed with brain cancer. While she was in the hospital, she learned about the Children’s Brain Tumor Foundation (CBTF) and soon became a volunteer with the organization. Today she is cancer free and a clinical social worker and regional coordinator at CBTF.

Regina also serves on a CIRM Clinical Advisory Panel (CAP) supporting a clinical trial for children with brain cancer. She says having a patient advocate like herself on the team to guide the clinical work of CIRM-funded research projects is vital to the success of clinical trials.

“I help researchers understand the needs of the patient, and why families don’t enroll in trials,” Karchner said.

“Among the families of kids with brain tumors we work with at CBTF, 80% are on Medicaid. It’s a select group of people who can afford to be in these trials; that’s why coming up with ways to make treatments affordable and accessible to all is so important.”



Photo: Stanford Medicine

Crystal Mackall, M.D.

Founding director of the Stanford Center for Cancer Cell Therapy, Crystal Mackall, M.D., is running a clinical trial that is testing a treatment for glioma, a devastating brain tumor that occurs primarily in children and young adults. Glioma tumors are almost always fatal.

Until now, radiation therapy has been the main treatment option, but it only extends survival by a few months. Dr. Mackall and her team are taking a revolutionary approach to treating blood cancers and adapting it to develop a next generation approach to be effective against untreatable brain cancers. They are modifying a patient’s own T cells (immune system cells) with a protein called chimeric antigen receptor cell (CAR). These newly created CAR-T cells will be re-introduced back into patients where it is hoped they will identify and destroy the brain tumor cells.



Photo Courtesy Nancy Ramos @ Silver Eye Photography

Regina Karchner, Patient Advocate and CIRM Clinical Advisory Panel member.

CALIFORNIA STATE UNIVERSITY FULLERTON



Empowering the Future for Everyone. Everywhere.

CIRM, the California Institute for Regenerative Medicine, offers extensive education opportunities to students of all ages, especially those from historically underserved communities. Samira Alwahabi, a recent college graduate and former participant in CIRM's Bridges program, is now a researcher at Stanford University. She plans to be a doctor and use her knowledge and cultural experience to improve patient care for everyone. **Everywhere.**

CIRM
CALIFORNIA'S STEM CELL AGENCY

www.CIRM.ca.gov