

Empowering Progress Through  
**Partnership**

2023 ANNUAL REPORT



# MISSION

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

At the California Institute for Regenerative Medicine (CIRM), we believe regenerative medicine can transform the lives of Californians suffering from diseases and disorders that currently have no known cure.

We are working tirelessly to make new treatments a reality by investing in innovative research, educational programs, and infrastructure to accelerate these discoveries.

The voters of California believed in this vision when they passed the California Stem Cell Research and Cures Act (Proposition 71) in 2004 to establish CIRM as the first state-funded institute to advance science for the people. This vote of confidence has allowed us to invest in the most promising therapies and empower the state's brightest minds to prepare for careers in regenerative medicine where they can push the boundaries of what is possible.

The passage of the California Stem Cell Research, Treatments, and Cures Initiative (Proposition 14) in 2020 renewed our funding and ensured our work could continue.

With \$5.5 billion slated for new investments, and more than 306 active stem cell and gene therapy research projects, we honor the trust the people of California have placed in us and strive continuously to fulfill our mission of delivering transformative regenerative treatments in an equitable manner throughout our Golden State.

CIRM funds cutting-edge research in regenerative medicine at academic institutions and biotechs across California.

*Photo: UCLA Broad Stem Cell Research Center*



# Contents

Welcome Letters. ....	2
Strategic Vision .....	3
By the Numbers .....	4
Diversity, Equity, and Inclusion .....	6
Access and Affordability .....	8
Research .....	10
Education .....	18
Infrastructure .....	22
Industry Partnerships .....	24
Administrative Update .....	27

## Regenerative Medicine: Empowering Progress Through Partnership

We envision a future where diseases can be treated using the power of stem cell and gene therapies. We believe that every Californian, regardless of their background or socioeconomic status, should have access to these life-changing treatments.

This vision is becoming a reality through the transformative potential of regenerative medicine—a field focused on reversing the course of disease by renewing and replacing damaged cells throughout the body, either by using gene therapy to correct underlying defects that lead to disease, or through other scientifically sound ways to recruit the body’s own repair mechanisms to heal tissues or organs.

By funding some of the best regenerative medicine research in the world, we are paving the way to address serious health issues in children and adults that have no known cure today—like cancer, diabetes,

fatal genetic diseases, neurologic disorders, sickle cell disease, and heart conditions.

But our vision goes beyond just developing new treatments. We believe that regenerative medicine has the power to transform healthcare as we know it, allowing us to move away from the traditional model of treating symptoms and instead focus on restoring health and function. This work has the potential to improve health outcomes and address debilitating conditions that have disproportionate effects on the underserved and underrepresented communities across our diverse state.

By collaborating with patient advocates, communities, academia, and industry, we already have made progress toward unlocking the full potential of regenerative medicine and creating a brighter, healthier future for all.

# Welcome Letters

## Dear Friends,

As we reflect on a successful year, it's clear that strong partnerships continue to play a pivotal role in achieving our ambitious strategic goals of accelerating world class science for California and the world.

This past year exemplified the importance of collaboration with scientists, healthcare professionals, manufacturers, students, patient advocates, industry groups, and community leaders to advance the field of regenerative medicine in the Golden State.

We've kept diversity, equity, and inclusion (DEI) at the forefront of everything we do, launching new educational programs to train a diverse workforce, and advancing efforts that address access and affordability.

We achieved all this while continuing to fund the most promising stem cell and gene therapy research to help address unmet medical needs of patients across a wide range of diseases and conditions.

In addition, we worked with communities across California to ensure that diverse voices have an opportunity to shape the future of CIRM programs. This report shares some of their stories.

We recognize there's still work to be done, but with our partners alongside us, we can continue to make great strides on behalf of all Californians.

Sincerely,



Maria T. Millan, MD  
President and Chief Executive Officer  
California Institute for Regenerative Medicine



## Dear Fellow Californians,

As my 12-year tenure as Chairperson of CIRM has drawn to a close, I reflect with a profound sense of accomplishment about all that CIRM has done to advance our vision to serve patients with unmet medical needs.

Having now deployed billions towards more than 1,300 grants addressing many diseases, CIRM is leaving an increasing, indelible mark on the fields of stem cell and gene therapy research.

Partnerships make this progress possible. Our partner stakeholders unfailingly offer input and participation in countless ways. Collectively, they have informed the far-reaching programs CIRM has developed over the years to advance the field.

In the early days, these included Alpha Clinics, the iPS cell bank, the Genomic Centers of Excellence, and more. That creative spirit carries on today in the form of new programs in manufacturing, data sharing, Community Care Centers of Excellence, and the initiative to address neurological disorders through research for some of humanity's most intractable health conditions.

It has been my true privilege to chair this effort all these years. I look forward to watching CIRM achieve even greater heights in the future.

In good health,



Jonathan Thomas, PhD, JD  
Former Chair  
Independent Citizens' Oversight Committee



# STRATEGIC VISION

Since CIRM's inception in 2004, we've deployed \$3.95 billion to fund regenerative medicine research, infrastructure, and education programs. CIRM advances regenerative medicine in California through collaboration, innovation, and support for all stages of research. We aim to provide timely access to regenerative medicine treatments, address manufacturing challenges, and promote inclusivity and diversity in the field.

Everything we do revolves around three strategic principles:

**Advancing**  
World Class Science

**Delivering**  
Real World Solutions

**Providing**  
Opportunity for All

Photo: UCLA Broad Stem Cell Research Center



Photo: VisionOne Studios, Jag Dhadiala



CIRM supports scientific research, infrastructure, and education programs to address unmet medical needs and create opportunities for Californians.

# Progress by the Numbers

As of June 30, 2023

## 9 Alpha Clinics

CIRM now supports nine medical facilities with staff experienced in cutting-edge clinical trials in regenerative medicine therapies

## 11 RMAT Designations

CIRM-funded projects recognized by the FDA as Regenerative Medicine Advanced Therapies to expedite development and FDA approval

## \$24+ billion

Investments in CIRM-funded research from biotechnology and pharmaceutical companies and other industry partners

## 410 Therapy Development Advisory Meetings

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

## 40+ Diseases/Conditions

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

## 50+ New Businesses

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

## 91 CIRM-Funded Clinical Trials

Clinical trials are vital steps on the path to U.S. Food and Drug Administration (FDA) approval of new therapies for patients

## 200+ Clinical Trials Conducted at Alpha Clinics

Hosting clinical trials for CIRM-funded research, as well as other research

## 3,400+ Participants in Clinical Trials

Pioneers who have consented to be part of a CIRM-funded clinical trial

## 3,800+ Interns and Fellows

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



Photo: UCLA Broad Stem Cell Research Center

## The Potential for Suppressing Seizures in a Single Dose

Annette Adkins (left) has experienced a remarkable transformation in her life since participating in a clinical trial funded by CIRM. The trial focused on testing the potential of neuronal cell therapy NRTX-1001 as a treatment to suppress seizures in patients with drug-resistant epilepsy.

In the past Annette endured frequent seizures, but thanks to this groundbreaking single-dose therapy, she is now hiking again and doing other activities she loves. Annette's story underscores CIRM's dedication to investing in meaningful research that provides real-world solutions for unmet medical needs.

*OHSU/Christine Torres Hicks*



Neurona Therapeutics, led by **Cory Nicholas, PhD**, received a CIRM grant to test NRTX-1001, a neural cell therapy derived from human stem cells, for drug-resistant epilepsy. In a small study, initial results showed a 90% reduction in seizures, improved memory scores, and no serious side effects, indicating that a single dose of NRTX-1001 could potentially suppress seizures long-term.

*Photo: Neurona Therapeutics/Curtis Myers*

# Diversity, Equity, and Inclusion Leadership

CIRM promotes the values of diversity, equity, and inclusion (DEI) in all programs we support, including our research, education, workforce development, and infrastructure programs. All people in California, regardless of their background, deserve equal access to the benefits of regenerative medicine.

## We champion diversity, equity, and inclusion...

### In patient outreach and clinical trial recruitment

We require applicants to submit a DEI plan showing how they will engage and work with underserved populations to include clinical trial participants from these communities. The plans also must outline the applicant's strategies to foster cultural sensitivity within their teams and partner institutions.

### In education and workforce development

CIRM provides valuable educational opportunities in regenerative medicine for students and young professionals from backgrounds that reflect the diverse communities of the state. Hands-on research and structured mentorships, such as the COMPASS program for undergraduates that launched this year, empower aspiring scientists to pursue rewarding careers in regenerative medicine.

### In research designed for a diverse California

Access to diverse cell lines for research can have far-reaching impacts on equitable health outcomes for Californians. We've created the world's largest single repository of human induced pluripotent stem cells (iPSCs), containing over 2,600 cell lines from individuals of diverse ancestries.



*The iPSC repository supports research on regenerative medicine and the impact of genetic diversity on diseases and treatments. Included cell lines represent African, Hispanic, Native American, East Asian, South Asian, and European ancestries. With access to such a comprehensive range of cell lines, researchers we fund can better understand and address the unique needs and genetic variations among different populations.*



“

DEI in medical research is essential to ensuring that the therapies we help advance will reach all patients in need, particularly in communities that are disproportionately affected and historically underserved.”

■ **Maria T. Millan, MD**  
*CIRM President and CEO*

CSU Fullerton student Ramadhan Ahmed was an intern at Stanford University through the CIRM-funded Bridges to Stem Cell Research and Therapy Program. Now, he's the first in his family to earn a college degree and is preparing for his first year of medical school at The Johns Hopkins School of Medicine with a full-ride scholarship. Here Ramadhan discusses research findings with CIRM CEO Maria T. Millan (left).

# Making Regenerative Medicine Reachable

CIRM works to make regenerative medicine accessible and affordable for all Californians, especially those from diverse and underserved communities. Together, we're shaping a more inclusive and equitable healthcare landscape in California, where groundbreaking regenerative medicine is within reach for all who need it.

CIRM's Accessibility and Affordability Working Group leads these efforts. The group sets strategies to ease clinical trial costs and collaborates with policymakers, regulators, and insurers to break down barriers and improve access to regenerative medicine treatments funded by CIRM.

## More Access to Clinical Trials

CIRM funds nine Alpha Clinics across California. These first-of-their-kind specialized clinics provide access to clinical trials and pave the way for groundbreaking treatments emerging from CIRM-funded research.

## Aligning to Community Needs

Listening sessions in communities across the state are providing broad insight into access and affordability challenges. CIRM is using this information to guide the development of Community Care Centers of Excellence that can respond to the stated needs, particularly in underserved communities.

## Supporting Patients

Our Patient Support Program will help Californians access CIRM-funded clinical trials and provide financial support through CIRM's Patient Assistance Fund. The program will also help remove financial barriers to participation in clinical trials. The current \$15.6 million fund will grow through future proceeds that result from revenue sharing commitments of CIRM funding.



It's about creating community partnerships ... it brings the community toward the science that we are trying to bring to them."

■ **Ysabel Duron**  
*CIRM Board Member*



# Forging Partnerships with Community Leaders

Community partnerships across California help CIRM meet patients where they are—literally. Leticia Olvera-Aréchar is the program manager at El Sol Neighborhood Educational Center, a community-based organization that provides services and resources in California’s Inland Empire. Leticia participated in CIRM’s Palm Desert and Sacramento listening sessions, to support

CIRM’s development of Community Care Centers of Excellence. She highlighted the importance of promotores, community-based health educators, and other community health workers who have built trust with people of diverse cultures in their communities. Leaders like Leticia can help shape the future of CIRM programs so they reach more people across California.



Promotores speak the same language and understand the community. We knock on doors—we go where the community is.”

■ **Leticia Olvera-Aréchar**  
*Promotora*



# The Stages of Medical Research

CIRM funds regenerative medicine research in three main stages that build upon one another: Discovery, Translational, and Clinical. This fiscal year, CIRM approved funding for 93 research projects, including research and development, as well as education (page 20) and infrastructure (page 22) initiatives that make research possible.

**33**  
PROJECTS  
APPROVED  
FOR FUNDING  
2022-2023



## DISCOVERY RESEARCH

Discovery is the initial stage of scientific investigation, where researchers aim to generate new knowledge through basic research by testing new ideas and using cell cultures and other tools to understand how diseases develop. Basic foundational early discovery research is especially important; this year we awarded 17 of our newest discovery program awards, DISCO.

**15**  
PROJECTS  
APPROVED  
FOR FUNDING  
2022-2023



## TRANSLATIONAL RESEARCH

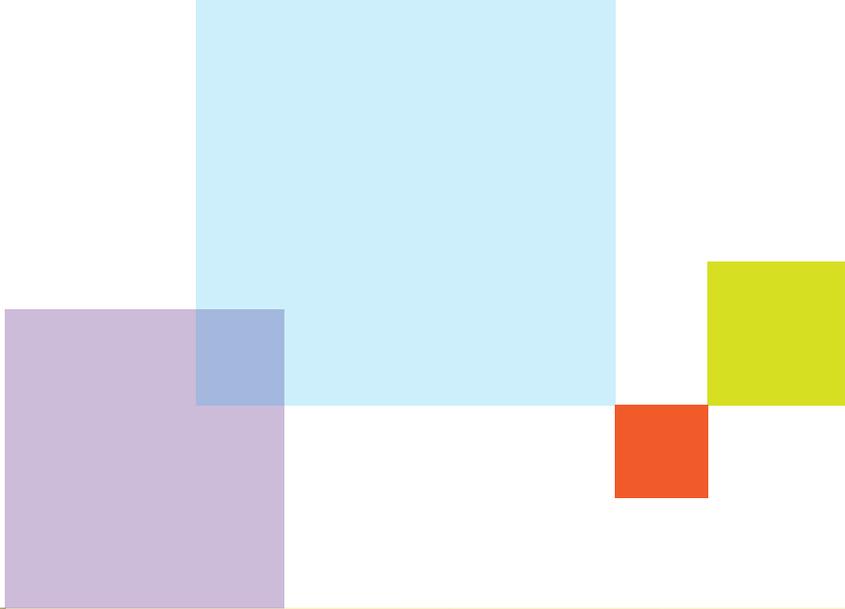
Building on knowledge gained during discovery, researchers next seek to “translate” research into new treatments that can eventually be tested in humans. This work explores whether the drug or therapy can be manufactured in a safe and predictable way, and that the treatments do not cause undue risks.

**22**  
PROJECTS  
APPROVED  
FOR FUNDING  
2022-2023



## CLINICAL RESEARCH

Once treatments have been tested in the lab, they advance to clinical trials to ensure they are safe and effective for people. Multiple trials are typically needed, and are a vital step before a new treatment can be approved by the FDA and made available to patients.



*Scientists study diseases in the laboratory to understand how they develop.*

**Julia Kaye, PhD**, at Gladstone Institutes, received a CIRM Discovery award to support research on how Amyotrophic Lateral Sclerosis (ALS, also known as Lou Gehrig's disease) develops. Her research uses patient-derived induced pluripotent stem cells, which are manipulated and analyzed to help discover the causes of ALS.

*Photo: Gladstone Institutes*



*Researchers aim to translate laboratory discoveries into new therapies to determine potential for human patients.*

**Preet M. Chaudhary, MD, PhD**, University of Southern California (USC), is developing a cell therapy product that targets prostate cancer cells. When expressed on the surface of immune cells, the product may bind to a certain protein that is overexpressed on prostate cancer cells in order to kill the cells.

*Photo: Keck Medicine of USC/Ricardo Carrasco III*



*Clinical trials involve testing treatments on people to ensure safety and effectiveness.*

**Thomas Chalberg, PhD** and CEO of Genasence Corporation, is the Principal Investigator of a gene therapy that aims to ease osteoarthritis symptoms. Up to 50 osteoarthritis patients will be enrolled in the clinical trial to see if the therapy reduces inflammation and pain, and potentially slows or reverses disease progression.

*Photo: Genasence Corporation*

# Innovation, One Patient at a Time



Not many four-year-olds are medical pioneers, but Hataalii Tiisyatonii (“HT”) Begay is in that small but mighty group.

Shortly after his birth in a remote part of the Navajo nation, HT was diagnosed with Artemis-SCID (Severe Combined Immunodeficiency Disease). Children born with the condition have no functioning immune system, so even a simple infection can be life-threatening or fatal.

But today, thanks to a therapy funded by CIRM and developed by researchers at the University of California, San Francisco (UCSF), HT is an energetic and healthy little boy who is home and off his medications.

HT was the first child to participate in the UCSF trial where lead investigators Morton J. Cowan, MD and Jennifer M. Puck, MD collect the patient’s own blood stem cells, modify them with a healthy version of the defective gene, and then re-infuse the corrected cells back into the patients. The genetically corrected cells then create a new, healthy blood supply and a functioning immune system.



Above, Dr. Jennifer M. Puck dances with HT, a young patient born with a rare genetic mutation. Right, Dr. Morton J. Cowan holds HT during his visit.

Photos: Barbara Ries for UCSF



Above, Child Life Specialist Courtney Dellinges comforts HT during a blood draw performed by Stella Gorin, RN.

“

We're pioneering gene therapy in this very rare disease right now, but we are using techniques that can be exported to heal many other conditions. Every new innovation happens one patient at a time.”

■ Jennifer M. Puck, MD  
UCSF



# The Interconnected Research of Rare and Prevalent Conditions



Photo: UC Regents, Courtesy of UC Davis

CIRM invests in both rare and prevalent disease research to uncover potential breakthroughs that benefit a wider population.

Addressing widespread diseases tackles significant public health challenges and costs. Investigating rare diseases, defined as impacting fewer than 200,000 Americans, presents opportunities for impactful regenerative medicine treatments and

breakthroughs. Exploring their interconnectedness maximizes the impact of CIRM funding and may improve healthcare outcomes for more people.

About half of active CIRM-funded clinical research is in rare diseases, which collectively impacts about 30 million Americans. More than 90% of rare diseases have no known treatment or cure.

## CIRM-Funded Research Portfolio by Disease Area Fiscal Year 2022-2023

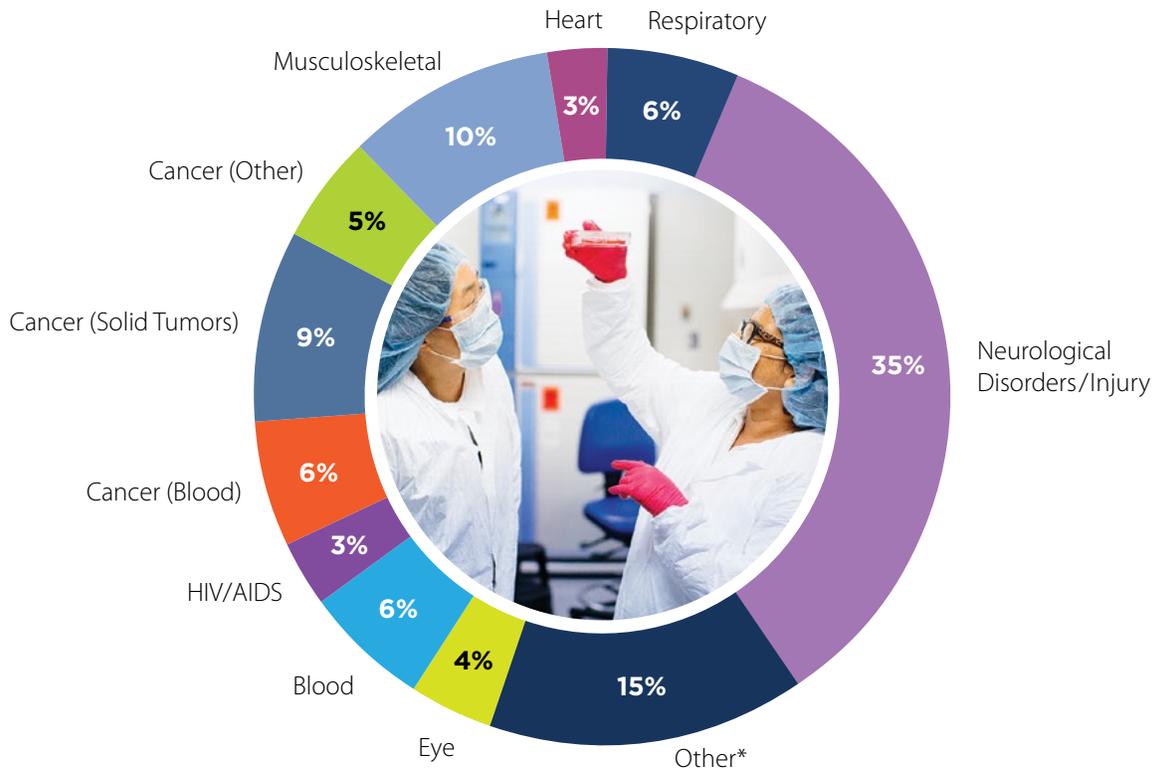


Photo: UCLA Broad Stem Cell Research Center

Percentages are normalized to the total number of Research awards approved during the 2022-2023 fiscal year. Research includes discovery, translational, and clinical research awards.

Percentages rounded to the nearest whole number.

\*Other includes: fertility, bladder, immune, and general stem cell biology.

## New Vision, New Hope

Veronica McDougall’s dreams were shattered by her diagnosis of retinitis pigmentosa (RP), a rare degenerative condition of the retina that would eventually leave her legally blind. She found new hope in a clinical trial funded by CIRM and led by Newport Beach-based biotech company jCyte. Her story showcases the powerful impact of innovative research fueled by strong collaborations like the one between CIRM and jCyte.

Veronica joined the clinical trial at the University of California, Irvine, where a dedicated research team injected retinal progenitor cells into her left eye, leading to steady vision improvement and some peripheral vision.

Although her vision deteriorated during her senior year of college, she joined a second CIRM-funded jCyte clinical trial, resulting in even stronger vision in her left eye. Veronica and her partner, Robert, now embrace parenthood with their son, Elliott. She hopes for another stem cell therapy to improve vision in her untreated right eye.



The doctors were partners with me in my journey. I don’t think I would have had the courage without them.”

■ **Veronica McDougall**  
Patient

## Partnering for Breakthrough Gene Therapies for Rare Diseases

CIRM joined the Accelerating Medicines Partnership Bespoke Gene Therapy Consortium last year to rapidly advance transformative gene therapies for rare diseases, which collectively affect millions of patients.



This important partnership between the National Institutes of Health (NIH), the FDA, more than 20 biopharma companies, and patient advocacy partners is building a blueprint to rapidly advance tailor-made—or “bespoke”—gene therapies to patients with rare genetic diseases.

CIRM demonstrates its commitment to this partnership by funding translational research led by Anthony J. Aldave, MD at the University of California, Los Angeles (UCLA) for Congenital Hereditary Endothelial Dystrophy (CHED), a corneal condition affecting children.

## Powering Progress

Since 2007, CIRM has funded \$933 million in discovery, translational, and clinical research projects to address Central Nervous System (CNS) disorders, including Alzheimer’s disease, Parkinson’s disease, ALS, stroke, dementia, epilepsy, depression, brain cancer, and autism.

CIRM has demonstrated a strong commitment to advancing scientific progress in the field of regenerative medicine, particularly in relation to CNS disorders. While a small portion (4%) of all grants were dedicated to researching the mechanisms underlying normal and disease

processes in the brain, CIRM’s strategic focus on funding these types of models have been instrumental in priming the field for significant advancements.

Discovery-stage research, which helps uncover new information about how diseases develop, is especially important in unraveling the mysteries of brain diseases and conditions.

CIRM’s Task Force on Neuroscience and Medicine is identifying potential areas where CIRM can make an impact in neurological disorders.

### PROFILE IN PROGRESS

#### Parkinson’s in the Spotlight

For Jenifer Raub, finding stem cell and regenerative medicine-based treatments for Parkinson’s disease is personal.

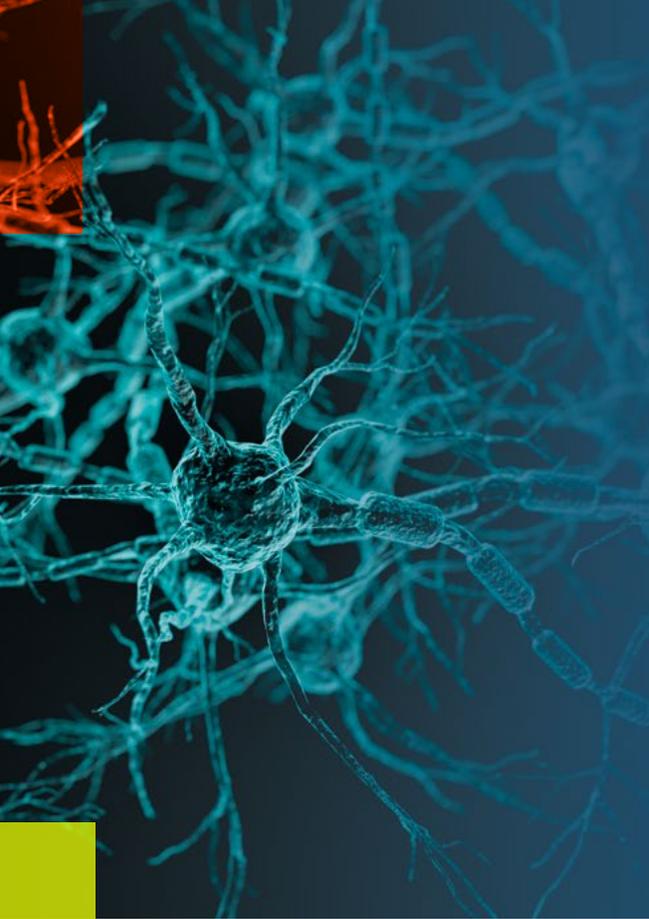
Diagnosed with Parkinson’s more than 15 years ago, Jenifer is a patient advocate and president of Summit for Stem Cell Foundation. She looks forward to the integration of regenerative medicine into mainstream healthcare for people with Parkinson’s and other neurodegenerative conditions.

She puts Parkinson’s in the spotlight whenever possible, whether it’s through fundraising, speaking on panels, or setting up booths at community events.



Answers need to be found. Not just for a cure, but for the cause, symptoms, and alleviation of pain. CIRM helps by funding research projects of all types for Parkinson’s...opening up new frontiers and possibilities that only exist in people’s dreams.”

**Jenifer Raub**  
*Patient Advocate*



## Collaborating to Find Treatments for Neurological Disorders

CIRM awarded \$4 million to San Diego-based Ryne Bio for a late-stage preclinical project aiming to improve treatment for Parkinson's disease, which impacts more than 100,000 Californians and one million people nationwide.

People with Parkinson's disease experience a decrease in dopamine-producing neurons, which leads to uncontrolled movements (dyskinesias) of the face, arms, and legs, as well as other effects such as dementia, depression, and sleep disorders.

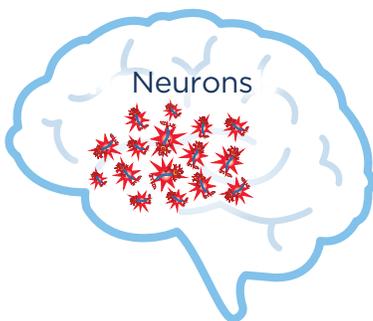
Investigators at Ryne Bio are aiming to deliver dopamine-producing neuron cells to replace those lost in the brain of Parkinson's patients to restore and improve motor function.

## A New Approach for Treating Parkinson's Disease

Dopamine-producing neurons in the brain help regulate body movement.

In patients with Parkinson's, dopamine-producing neurons become damaged and die, which leads to symptoms such as tremors, stiffness, and difficulties with balance.

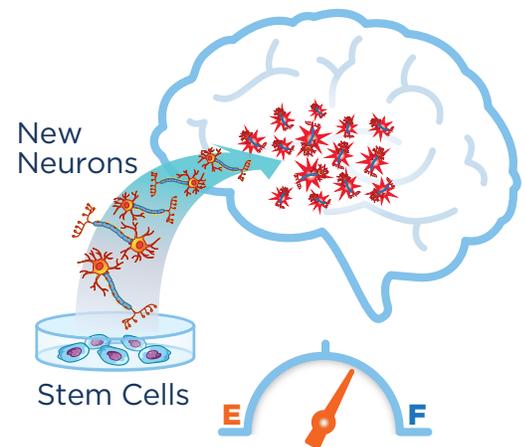
CIRM is funding research by Ryne Bio that uses stem cells to create new neurons to replenish damaged neurons in the brain with the goal of restoring and improving motor function.



Dopamine-Producing Neurons



Dopamine-Producing Neurons



Dopamine-Producing Neurons

# Cell and Gene Therapy Trailblazers

To bring regenerative medicine breakthroughs to California and the world, we need a dedicated and skilled workforce.

CIRM-supported education investments help students from high school to doctoral candidates. In partnership with California community colleges and universities, CIRM-funded education programs aim to recruit participants from diverse backgrounds and perspectives who can bring fresh and valuable ideas to the field of regenerative medicine. Participants attend specialized courses, engage with patients and communities, and gain hands-on



research experience in cutting-edge labs at universities and biotechnology companies across the state.

Eighteen new CIRM-funded education programs this year will support 500 interns and fellows over the next five years. Each new cohort empowers a new generation of Californians to rise as trailblazers in regenerative medicine.

# 18

EDUCATION PROGRAMS  
APPROVED FOR FUNDING  
2022-2023

A powerful vision fuels California's stem cell research endeavors. We unleash the potential within a talented workforce to propel scientific discoveries into life-altering solutions.

CIRM-funded education programs include hands-on internships and fellowships that culminate in student-conferences for learning and networking.



## Kevin Brown's Boundless Horizons

Kevin Brown participated in the CIRM-funded Bridges to Stem Cell Research and Therapy Program, which he credits for helping him find a career path that fulfills his passions for both exploration and patient care.

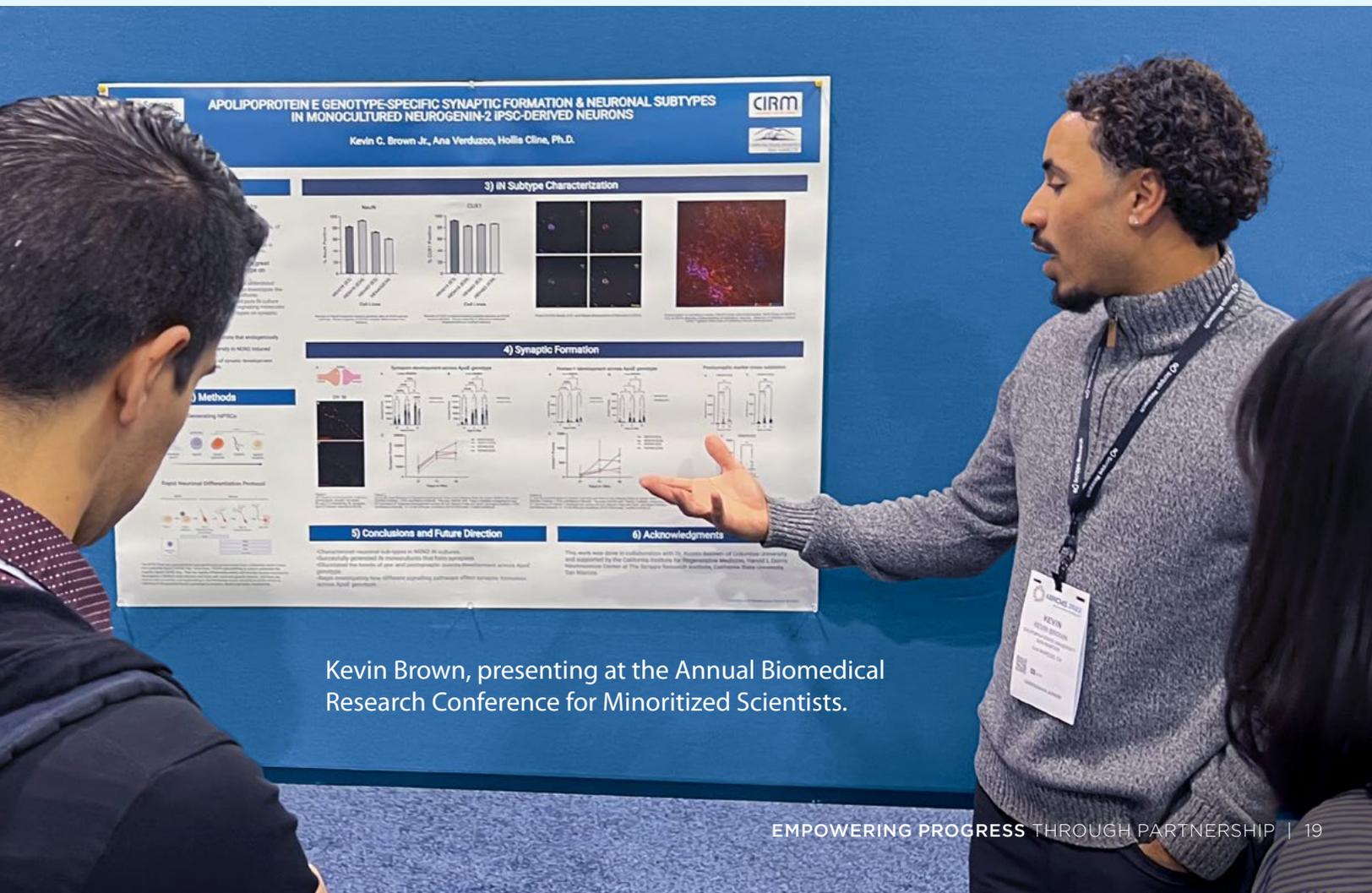
"As I got deeper into my research, I fell more and more in love with the process of science and began seeing myself as a leader in this field in the future. Stem cell research is vital to understanding how we can address diseases and conditions that impact humans," Kevin said.

At the Annual Biomedical Research Conference for Minoritized Scientists, Kevin won the 2022 Outstanding Presentation Award. Today, he is a full-time student at California State University, San Marcos, completing his Bachelor of Science in Biology with a concentration in Physiology. He



is still a part-time research intern at Scripps Research Institute.

Kevin also pays it forward for other students, building a mentoring network to help high school students participating in the CIRM-funded SPARK internship program.



Kevin Brown, presenting at the Annual Biomedical Research Conference for Minoritized Scientists.

# Education and Training Programs

## SPARK

*Summer Program to Accelerate Regenerative Medicine Knowledge*

# 11

PROGRAMS  
STATEWIDE

CIRM's paid summer internship program offers diverse high school students unique opportunities in stem cell research—particularly students who might not otherwise have the chance to take part in internships due to economic constraints.

### Rooster Garcia

INTERNSHIP: UCSF

"I've always wanted to conduct experiments and see day-to-day life in a lab. It was amazing to see the ins and outs of how a lab operates and how united each lab team member is with one another."



## BRIDGES

*Bridges to Stem Cell Research and Therapy*

# 15

PROGRAMS  
STATEWIDE

The Bridges fellowship and mentoring program helps prepare diverse and disadvantaged candidates for undergraduate and master's degrees in science for research and career opportunities in regenerative medicine.

### Elaine Lai

INTERNSHIP: UC Irvine



"I wanted to make a difference in both human health and science. The Bridges program helped me explore different ways stem cell research was being used to treat and study disease."

## COMPASS

*Creating Opportunities Through Mentorship and Partnership Across Stem Cell Science*

# 16

PROGRAMS  
STATEWIDE

The COMPASS undergraduate training program fills a pressing need to prepare and nurture a diverse population of undergraduate students for careers in regenerative medicine. It helps build a workforce that reflects California's demographics and is sensitive to disparities in research and healthcare.

### Madison Pierce

**INTERNSHIP:** University of Southern California

"With a chronic medical condition of my own, I have always wanted to help improve the quality of life for those with diseases and disabilities. Regenerative medicine research helps me take this goal to the next level."



## CIRM SCHOLARS

*Training in Discovery and Translational Regenerative Medicine Research*

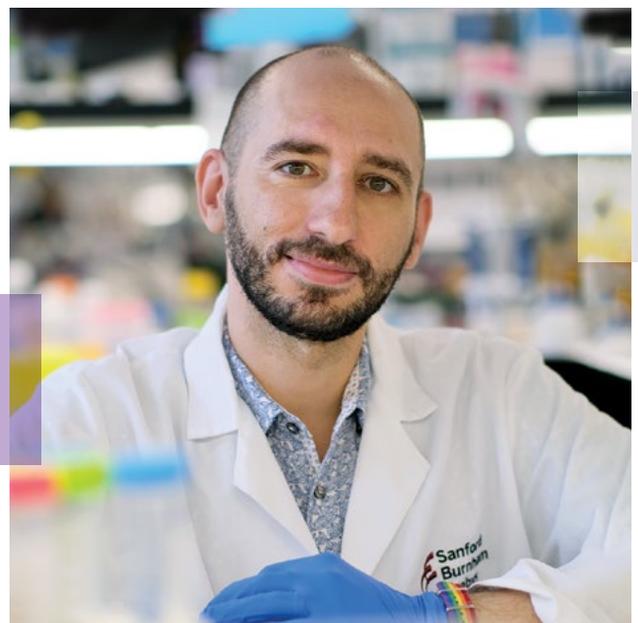
# 18

PROGRAMS  
STATEWIDE

The CIRM Scholars program supports and trains California scientists at various educational stages, including pre- and post-doctoral and clinical fellow levels. These scholars contribute to the expansion of stem cell research as they become skilled researchers and aspiring regenerative medicine leaders.

### Luca Caputo

**FELLOWSHIP:** Sanford Burnham Prebys Medical Discovery Institute



"The opportunity to be a CIRM postdoctoral fellow provides the necessary time and resources to complete my proposed research and facilitate my career transition into an independent investigator."

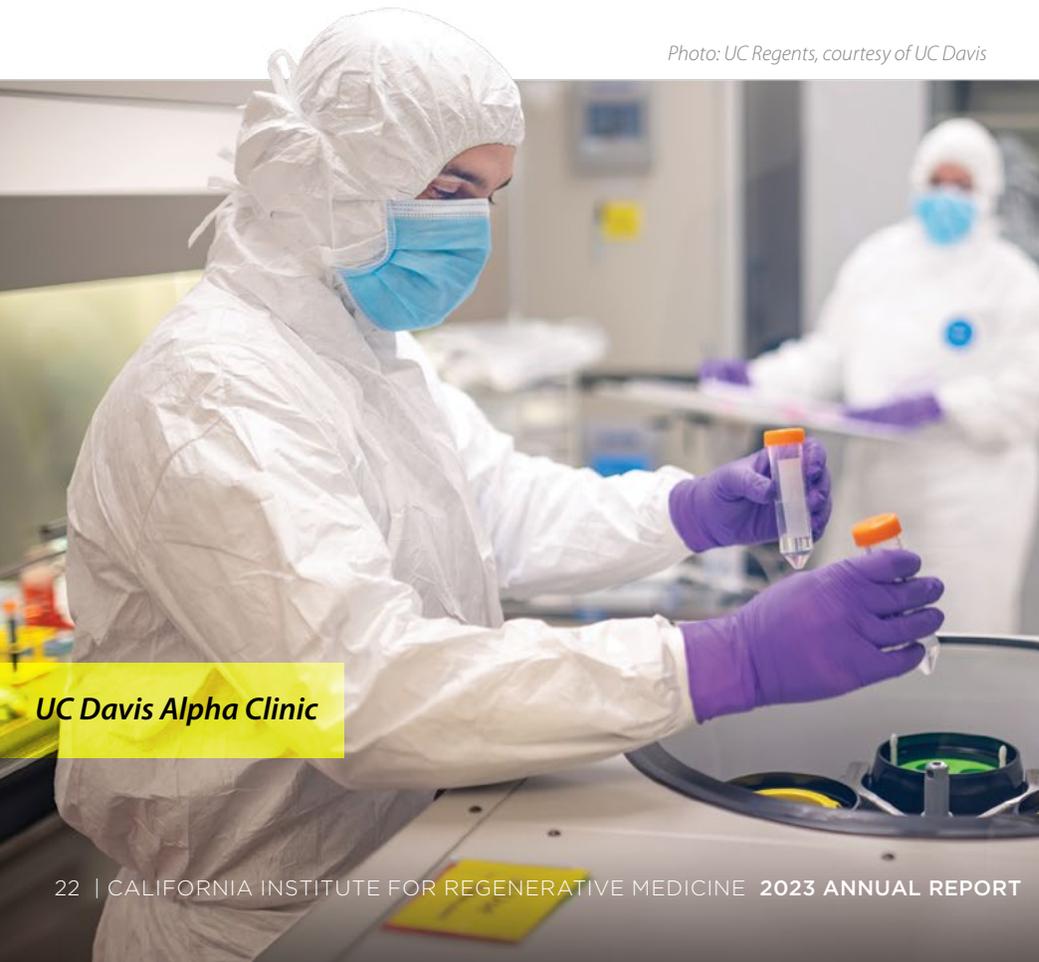
# Infrastructure That Ignites World Class Science

CIRM has ambitious goals to foster an open and collaborative culture among scientists, break down barriers, and build a culture of “team science” to empower and connect California’s research ecosystem.

## Alpha Clinics

The CIRM-supported Alpha Clinics Network now boasts nine leading medical centers throughout California. The network specializes in delivering stem cell and gene therapy clinical trial opportunities and treatments to patients. The network supports both CIRM-funded clinical trials and those funded by academic and industry sponsors. Recognizing the Alpha Clinics Network’s success, the CIRM governing board approved an \$80 million investment to expand the network by four sites in 2022.

Photo: UC Regents, courtesy of UC Davis



UC Davis Alpha Clinic

**Alpha Clinics Network:**  
Accelerating Regenerative  
Medicine to Californians

**200+** CLINICAL TRIALS

**1000+** PARTICIPANTS

**40+** DISEASE AREAS

Stanford University

University of California,  
San Francisco

Cedars-Sinai Medical Center

City of Hope

University of California,  
Davis

University of California,  
Los Angeles

University of Southern  
California / Children’s Hospital  
Los Angeles

University of California, Irvine

University of California,  
San Diego



Shared resources, including state-of-the-art lab equipment and expertise, foster collaboration (above).

Dr. Rajni Agarwal-Hashmi (right), a professor of pediatrics at Stanford University and member of the Stanford Alpha Clinic's outreach working group, meets with a patient.

*Photo: UCLA Broad Stem Cell Research Center (top)*

*Photo: Stanford Medicine (right)*

---

## Shared Labs

CIRM-funded Shared Resources Laboratories for Stem Cell-Based Modeling will support statewide academic and nonprofit research institutions by helping them provide access to cutting-edge lab equipment, essential resources, and comprehensive research training. The goal is to break down research silos, foster collaboration, reshape research culture, and provide students and researchers access to top-notch resources and training. CIRM began accepting applications for shared resources grants in summer 2023.



---

## Knowledge Networks

Sharing data, knowledge, and ideas helps scientists build on knowledge gained by others to advance the field of regenerative medicine. CIRM is helping fund the infrastructure needed to make it easier to share data and knowledge.

As a first step, CIRM has made great progress in advancing Knowledge Networks by implementing a new program for Data Sharing and Management. In alignment with the National Institutes of Health,

CIRM requires grantees to develop and implement Data Sharing and Management Plans. CIRM grantees will share data that researchers across California and the world can access and leverage in their own studies.

---

*CIRM also has established a **California Cell and Gene Therapy Manufacturing Network** to address bottlenecks and scalability challenges. Details on page 25.*

# Accelerating Public-Private Partnerships

Collaboration is crucial on the long and costly journey to advance breakthrough therapies. Private investment in CIRM-funded projects has exponentially accelerated vital research, bringing life-changing treatments closer for California patients.

## Industry investment as a measure of success

CIRM's early involvement in cell and gene therapy research signals reduced risk and attracts robust follow-on private investments — \$24.4 billion to date. CIRM-funded projects continue attracting industry investments and partnerships, despite funding uncertainties related to difficult market conditions. For example, CIRM's early research funding for Ray Therapeutics' gene therapies for blinding diseases helped the company attract \$100 million in venture capital.

## Partnerships for progress

Launched in 2022, CIRM's Industry Resource Partners (IRP) network promotes collaboration and resource sharing. IRP members provide cost-effective services, technologies, and expertise to CIRM-funded projects. The first IRP member, global healthcare company Novo Nordisk, offers its proprietary stem cell lines and support in translating research into practical patient treatments.

## Supporting early-stage stem cell research

CIRM has worked with five additional industry partners to make clinically compatible stem cell lines available for CIRM-funded discovery-stage research. Enabling access to these cell lines accelerates development of regenerative medicine therapies and promotes equitable health outcomes for Californians.

San Diego-based National Resilience, Inc. has committed to providing CIRM-funded researchers and applicants with project consultation, and access to manufacturing resources for cell therapy, gene therapy, and biologics.



Novartis, one of the largest pharmaceutical companies in the world, acquired the rights to a CIRM-funded gene therapy for cystinosis from AVROBIO for \$87.5 million in May 2023. CIRM is currently funding the phase 1/2 clinical trial at UC San Diego.

## Overcoming Manufacturing Hurdles

This year CIRM launched the California Cell and Gene Therapy Manufacturing Network to help de-risk the development and approval of cell and gene therapies, and to expand the manufacturing workforce in California.

In the initial stage, CIRM is funding five non-profit facilities in California that follow good manufacturing practices (GMP) to produce regenerative medicine therapies for preclinical

testing and clinical trials. The funding will help these essential facilities improve manufacturing quality and efficiency and develop cutting-edge technology platforms.

CIRM-funded GMP facilities will collaborate with community colleges and universities in California, including CIRM educational program awardees, to prepare individuals for manufacturing careers.



A laboratory at UCLA's current good manufacturing practices facility.

*Photo: Elana Zhukova, courtesy of UCLA*

## Moving Faster: Advancing Research to Treat Type 1 Diabetes

Katy Digovich has been living with Type 1 diabetes (T1D) for over 25 years.

With support from a \$1.2 million Discovery research grant from CIRM, Katy and her team at Minutia, Inc., in Berkeley are working to produce a minimally invasive transplant of insulin-producing cells combined with nanosensors—tiny particles that can detect biomarkers in cells related to transplant assaults and success.

The goal is to replace lost insulin-producing cells in the pancreas of patients with T1D for cell replacement therapy, and also to reduce transplant variability, and improve outcomes.

In the United States, 1.6 million adults aged 20 years or older have T1D and require insulin. Minutia's product would be delivered under the skin to adult patients with T1D, and if successful, could revolutionize care by reducing or eliminating the need for continuous insulin therapy.



CIRM has been an invaluable partner. CIRM's funding, as well as advice and guidance, have already helped Minutia move faster as we advance our therapy."

**Katy Digovich**  
CEO, Minutia, Inc.



## About Our Governing Board

The Independent Citizens' Oversight Committee (ICOC) is CIRM's esteemed governing board, comprising experts from scientific, medical, and patient advocacy backgrounds. With 35 members, the ICOC makes final decisions on awarding CIRM research grants, along with other priorities including raising awareness, building public trust, and highlighting CIRM's role in regenerative medicine.

In March 2023, Vito Imbasciani, PhD, MD, became the ICOC Chair, leveraging his expertise

in medicine, government, and advocacy to champion CIRM's objectives.

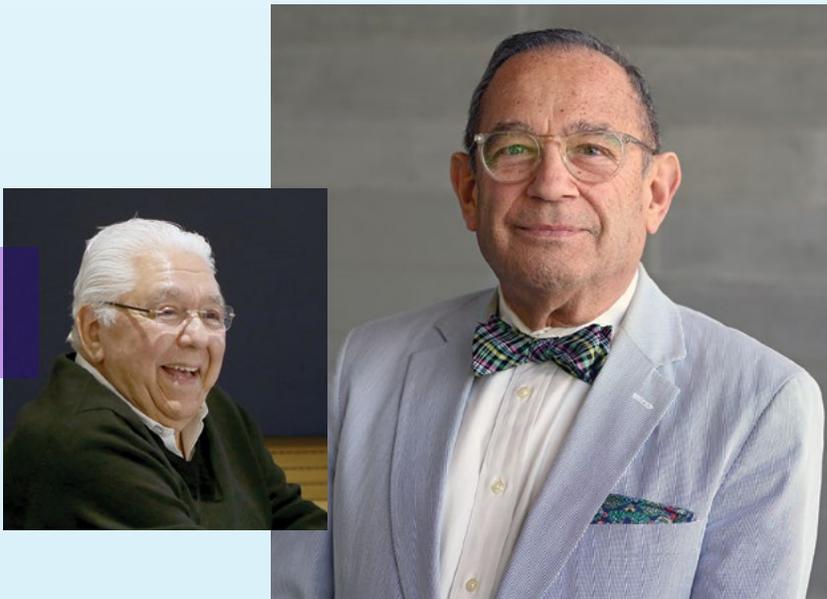
In January 2023, Maria Gonzalez Bonneville, with over a decade of experience in public outreach, board governance, and communications at CIRM, was appointed Vice Chair.

The departure of Chair Jonathan Thomas, PhD, JD, and Vice Chair former Senator Art Torres was marked with deep gratitude for their 12 years of service, during which time their passion, commitment, and integrity was instrumental in guiding CIRM's success.



I look forward to the challenge of advancing the groundbreaking work of CIRM, at the same time nourishing the hopes for medical advances held by the citizens of our great state.”

■ Vito Imbasciani, PhD, MD  
*ICOC Chair*



Former ICOC Vice Chair Art Torres (left). Incoming ICOC Chair Vito Imbasciani, PhD, MD, (center) and Maria Gonzalez Bonneville, being sworn in as ICOC new Vice Chair (right).

# 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 2022-2023: \$426.7 million

Funds Approved*	\$ 304.3 million
Funds Remaining	\$ 96.3 million
Balance Under Active Management	\$ 1.2 billion
Number of Active Project Awards	306

\*Does not include allocated budget of \$26.2 million for pending awards for Discovery and Conference Grants

## Approved Budget for Fiscal Year 2023-2024: \$486.3 million



### Discovery Research

\$84.7 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 Research

\$84.6 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 Research

\$252 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.



### Education Programs

\$2.5 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.



### Infrastructure Program

\$62.5 million

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

# Our Team: Building Partnership

At CIRM, we actively seek partnerships with scientists, industry leaders, patient advocates, academic institutions, and communities across California to build a diverse research ecosystem that drives scientific progress. Just as important, CIRM fosters a culture of internal collaboration to accelerate research and development of

regenerative medicine and advance our mission. By fostering partnerships within and outside the organization, CIRM harnesses the collective expertise, resources, and perspectives necessary to drive the field of regenerative medicine forward and create innovative breakthroughs that benefit California and the world.



## IN MEMORIAM

We dedicate this annual report to Kevin McCormack, who passed away unexpectedly in December. As CIRM's Director of Patient Advocacy, Kevin personified the power of partnership.

He made sure patients were seen and their stories heard. He built a platform for the patient advocacy community to amplify our shared passion for medical breakthroughs. He brought hope for a better future for everyone.

CALIFORNIA  
INSTITUTE FOR  
REGENERATIVE  
MEDICINE



Back Cover Photos: Denise Penagos, UCLA Broad Stem Cell Research Center, Sarah White of SDSU, Gillian Grisman UCSF

Front Cover Photos: Barbara Ries for USCF, UCLA Broad Stem Cell Research Center, Cal State Fullerton, UCLA Broad Stem Cell Research Center

**CIRM**  
CALIFORNIA'S STEM CELL AGENCY

601 Gateway Blvd, Suite 400  
South San Francisco, CA 94080  
(510) 340-9101 | [www.cirm.ca.gov](http://www.cirm.ca.gov)

