CIRM

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

20TH ANNIVERSARY

2024 ANNUAL REPORT

OUR JOURNEY FORWARD: HOW CIRM CATALYZES REGENERATIVE MEDICINE TODAY AND BEYOND

Mission

CIRM's mission is to accelerate world-class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world.

Twenty years ago, Californians approved Proposition 71 to establish the California Institute for Regenerative Medicine (CIRM), the first state Agency dedicated to advancing cutting-edge stem cell research and therapies.

This support launched CIRM's mission to deliver transformative therapies to Californians, and in doing so, catalyzed the regenerative medicine field throughout the state. The passage of Proposition 14 in 2020 renewed CIRM's funding for another $5.5 billion, allowing the Agency to continue its important work to address diseases with high unmet medical needs.

Over this time, CIRM has accelerated the development of groundbreaking treatments and nurtured California's biotech ecosystem, encompassing hospitals and clinics, manufacturing sites, and vital industry collaborations. Through these initiatives and programs, CIRM continues to speed up the path from scientific discovery to patient care.

As CIRM moves into its third decade, its promise remains: to accelerate regenerative medicine for the well-being of all Californians.

Photos: UCLA Broad Stem Cell Research Center

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The Power of Regenerative Medicine

Regenerative medicine is a rapidly advancing field that utilizes therapies involving stem cells and gene editing to improve patient outcomes. These innovative treatments have the potential to cure some of the world's most vexing diseases and conditions.

By funding the best regenerative medicine in the world, CIRM continues to drive this progress in treating diseases like cancer, diabetes, Parkinson's disease, genetic disorders, and more.

But it's not just about finding new treatments—it's about revolutionizing science and healthcare delivery.

Through its education pillars, CIRM empowers the brightest minds to unlock discoveries that address the root causes of illness. These approaches have the potential to make a significant impact, particularly for communities that have historically faced barriers to quality health care.

CIRM will continue to partner with scientists, patient advocates, industry leaders, and all other stakeholders to deliver on our mission.

Leadership Letters

Dear Fellow Californians,

2024 marks CIRM's 20th anniversary year as the State's landmark granting Agency in support of research into cell and gene therapies.This year, CIRM has reached a significant milestone by surpassing the 100 mark in human clinical trials.

We have launched initiatives such as the Patient Support Program (PSP) to broaden access to clinical trials and treatments. The Board has approved an expansive plan for the Community Care Centers of Excellence (CCCEs), which aim to expand access to CIRM-funded clinical trials and regenerative treatments across California.

CIRM will also expand the capacity of our existing collaborative network of specialized research laboratories on stem cell research.

This year, we introduced a new class of clinical grants that will advance a therapy to the point where it can be commercially licensed and available to the public.

So, while we look back proudly at what we have accomplished over the last 20 years, we are also laying the groundwork for continued innovation and success.

Sincerely,

Vito Imbasciani, PhD, MD, FACS
Chairman, Independent Citizens' Oversight Committee (ICOC)

Dear Fellow Californians,

As we approach CIRM's 20th anniversary, we take great pride in reflecting on our many accomplishments. Starting in 2004 when the stem cell field was in its infancy, CIRM has been a key player, enabling best-in-class research in the quest to develop therapies and cures. To date, CIRM has awarded over $3.86 billion in 1400+ grants across basic research, translational research, and clinical trials. This work has culminated in an important milestone at CIRM: the funding of 108 clinical trials at the close of the 2023-2024 fiscal year.

Our funding has strengthened California's biotech ecosystem, encompassing Alpha Clinics and manufacturing facilities. CIRM's industry partnerships continue to grow and our education and training opportunities are preparing tomorrow's scientists. Importantly, all of our programs are grounded in the principles of diversity, equity, and inclusion.

In recent months, demand for CIRM funds has grown by orders of magnitude. The increased demand, coupled with the fact that we have $3.8 billion left to deploy—$1.1 billion of which must go to research in neurological disorders per Proposition 14—has led us to undertake a number of initiatives that will set CIRM's agenda for the coming years.

Sincerely,

Jonathan Thomas, PhD, JD
President and CEO

Our Impact at a Glance

* As of June 30, 2024

CIRM's impact is evident through its significant contributions to the advancement of regenerative medicine. The following metrics highlight the Agency's achievements in supporting medical facilities, expediting therapy development, and investing in research.

9 Alpha Clinics

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

12 RMAT Designations

These CIRM-funded projects have been recognized by the FDA as Regenerative Medicine Advanced Therapies, a designation given to expedite development and FDA approval.

$24+ billion Industry Investments

This significant investment in CIRM-funded research comes from biotechnology and pharmaceutical companies, as well as other industry partners.

438 Therapy Development Advisory Meetings

These Translational and Clinical Advisory Panels have assisted CIRM-funded researchers in accelerating the development of their therapies and anticipating future challenges.

85+ Diseases/Conditions

CIRM funding helped advance 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.

108 CIRM-Funded Clinical Trials

By directing support for 108 clinical trials, CIRM plays a crucial role in advancing its mission to deliver innovative stem cell and gene therapies to patients.

250+ Clinical Trials Conducted at Alpha Clinics

Number of clinical trials for CIRM-funded research, as well as other research.

4,300+ Participants in Clinical Trials

Number of pioneers who have consented to be part of a CIRM-funded clinical trial.

4,317 Interns and Fellows

Number of high school and college students, post-doctoral candidates, and clinical fellows involved in CIRM-supported education and training programs.

Photo: Gladstone Institutes

Accelerating Groundbreaking Discoveries for Two Decades

2004
The Beginning

The California Stem Cell Research and Cures Act known as Proposition 71 is approved by California voters, establishing CIRM and authorizing $3 billion for stem cell research and therapy development in California.

2006
First Strategic Plan

CIRM's Board develops its first scientific strategic plan to help guide the disbursement of funds over a ten-year period.

2009
Bridges Internships for College Students

CIRM launches the Bridges Program, which provides paid research internships to students at universities and colleges without major stem cell research programs.

2004 | 2005 | 2006 | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014

2005
CIRM Announces First Request for Application

CIRM's Board approves the Agency's first Request for Application (RFA)—a training program for California's universities and non-profit academic and research institutions.

2008
Major Facilities Funded

A CIRM Major Facilities Grant Program funds the establishment of building facilities across the state to support stem cell research.

2012
High School Creativity Awards

CIRM launches the Creativity Awards Program to provide internships to high school students. This program is later renamed Summer Program to Accelerate Regenerative Medicine Knowledge (SPARK).

2014
Stem Cell Cure for "Bubble Baby" Disease (SCID)

A UCLA research team led by Donald Kohn, MD, announces a breakthrough gene therapy and stem cell cure for "bubble baby" disease, or severe combined immunodeficiency (SCID).

Photos: Sarah White / SDSU, Jaquell Chandler

2015
Alpha Clinics Network Created

CIRM's Board approves a $70 million plan to develop innovative Alpha Clinics across the state to support and accelerate stem cell clinical trials.

2015
CIRM 2.0 Unveiled

Under the leadership of then President C. Randal Mills, PhD, CIRM launches CIRM 2.0, a strategic plan that transforms its funding model to streamline and accelerate the advancement of stem cell research.

2019
Finding Treatments for Sickle Cell Disease

CIRM and the National Heart, Lung, and Blood Institute (NHLBI) create a landmark agreement to find treatments for sickle cell disease.

2024
100+ Clinical Trials

The Board approves five new clinical trials for Parkinson's disease, autoimmune conditions, cancers, and other conditions, bringing CIRM's clinical awards to over 100.

2015 | 2016 | 2017 | 2018 | 2019 | 2020 | 2021 | 20021 | 2022 | 2023 | 2024

2018
50th Clinical Trial

The Board approves CIRM's 50th clinical trial which was related to the toxic side effects of chemotherapy.

2020
Yes on Prop 14

California voters approve the California Stem Cell Research, Treatments, and Cures Initiative—also known as Proposition 14—which provides $5.5 billion in renewed funding to CIRM.

2021
Accessibility and Affordability Working Group Kicks Off

The Board approves a new strategic plan for 2022-2027 and commences its first Accessibility and Affordability Working Group (AAWG) meeting to evaluate strategies for making CIRM-funded therapies accessible to all Californians.

2023
Launch of the CIRM Neuroscience Task Force

The CIRM Neuroscience Task Force launches as part of Prop 14's mandate to allocate $1.5 billion toward the research and development of therapies for neurological disorders.

2023
ReMIND Program Approved

The Board approves a new plan to invest $110 million into basic research in neuropsychiatric diseases —one of the largest investments of its kind in California.

2023
Manufacturing Network Awards

CIRM establishes a new manufacturing network at its nine Alpha Clinics sites to reduce bottlenecks and accelerate pathways to commercialization for cell and gene therapies.

Progress at CIRM has been made possible through the dedication of patients, researchers, Board members, and stakeholders who have remained supportive of its mission throughout the years. Hear from them—in their own words—about CIRM's pivotal role in advancing regenerative medicine.

Jennifer Doudna, PhD, Nobel Laureate in Chemistry, Professor of Biochemistry at UC Berkeley, and Founder of the Innovative Genomics Institute

"CIRM has revolutionized regenerative medicine by funding hundreds of projects and supporting key clinical trials, significantly advancing the development of new treatments for diseases such as cancer, diabetes, and neurodegenerative disorders.

CIRM's most significant contribution is the creation of a robust infrastructure for stem cell research in California, which has accelerated the transition of groundbreaking fundamental research from the lab to clinical applications, benefiting patients worldwide. The future of regenerative medicine holds the promise of personalized therapies and potential cures for previously untreatable conditions.

George Daley, MD, PhD, Dean of the Faculty of Medicine, Harvard Medical School

"CIRM has catapulted the California stem cell community into international prominence. Numerous California-based scientists are now major leaders in the field of stem cell biology, owing in large part to the availability of flexible CIRM funding for projects that wouldn't or couldn't be funded by federal grants. We're now seeing positive data emerge for stem cell and gene therapies for leukemia, diabetes, blindness, and neurologic disease as the result of decades of patient investment in new technologies. One cannot overstate the importance that CIRM has played in the stem cell revolution.

Don C. Reed, Stem Cell Champion and Patient Advocate

"Built from a citizens' initiative, Proposition 71, CIRM expresses the will of California: that regenerative medicine should fight chronic disease and disability. One joyous victory was over Severe Combined Immune Deficiency. Thanks to this CIRM-funded therapy, 50 children have now survived the disease. Thanks to stem cells, young men and women have regained upper body strength and control, enhancing their lives. Therapies developed by CIRM will continue to benefit patients for generations to come. What a tremendous blessing, 20 years of effort!

Ysabel Duron, CIRM Board Member, Founder/ Executive Director of the Latino Cancer Institute, and Patient Advocate

California taxpayers should feel proud of CIRM's efforts and accomplishments. The leaders of the initiative 20 years ago were bold enough to take on this heavy lift, when the federal government stepped back. Not only did CIRM move to diversify the field, but it took a significant step to be sure that diverse communities were included in research and reaped the benefits of new findings and clinical trials. CIRM is at the forefront of making sure that regenerative medicine provides a level playing field so that all communities can move past hope to an opportunity to reap the rewards of these scientific discoveries.

Robert Klein, Initiative Author, Campaign Chair, CIRM Founding Chairman

November 2, 2004, California voters, by nearly a 60% approval, launched a new vision for funding breakthrough scientific research and clinical trials, to generate stem cell therapies, across a broad range of chronic diseases. The New York Times lead editorial, the next day, led with the title: "California to the Rescue". California's funding of $3 billion for stem cell therapies broke through the religious barrier paralyzing the federal government funding and challenged foreign countries, states, and universities—globally—to join the Stem Cell Medical Revolution. The California Institute for Regenerative Medicine, CIRM, has led this revolution, with proof of concept FDA Phase II therapies, restoring sight for patients with Macular Degeneration, freeing patients from insulin—for Type 1 Diabetes, providing initial evidence that Epileptics can be free from seizures, and contributing multiple breakthroughs in cancer therapy, resulting from national and international collaborations and progress built on its funded research and human trials. Now, with the California voters' $5.5 billion approval in 2020, CIRM—with an extraordinary, dedicated staff and California's amazing scientists—in collaborations across our nation and the world, is driving the Stem Cell Revolution, integrated with genetic therapies, towards the California vision of reducing the future of human suffering , in recoveries from chronic disease, with therapies that restore quality of life for children, for every family, for every citizen of our great state, and the global community. Let us give thanks, in celebration, for California's voters.

Rich Lajara, CIRM Board Member, Clinical Trial Participant, Spinal Cord Injury (SCI) Patient Advocate

CIRM has made many contributions to the field of science over the past 20 years. What stands out the most is the impact on the thousands of patients enrolled in CIRM-funded clinical trials over this time. Because I participated as patient #1 in a CIRM trial, I know how much of a selfless act it is to put yourself out into what seems to be the unknown. The data collected from each individual is priceless and imperative to advance the field of regenerative medicine. They are my true heroes for helping pave the path for cures for future generations. The future of regenerative medicine needs to be affordable and cost-effective. CIRM has a focused role to help ensure the future of potential treatments remains cost-effective and attainable.

Heidi Edwards, President and Founder of Sisters Hope Foundation

CIRM's work and dedication have led to groundbreaking research, discoveries, and cures for those diagnosed with devastating diseases such as Adult-onset leukoencephalopathy (ALSP). Thank you to CIRM for so much more than just financial support. Thank you for your drive, dedication, and passion for the past 20 years. Without amazing people like you, my family may have never seen a future of HOPE and a life without the devastation of ALSP.

Adrienne Shapiro, Patient Advocate and Co-founder of Axis Advocacy

In 2020, we advocated for more CIRM funding. I knew what it meant and how CIRM would change the world. My dream has come true because CIRM has been the catalyst for saving so many families from generations of pain and suffering, not only from sickle cell disease but also from so many other known and unknown diseases.

It has been a little over 20 years since Bob Klein and others identified this need and began this revolution in California. I cannot wait to see what happens in the next 20 years.

Sandra Dillon, Clinical Trial Participant, previously diagnosed with myelofibrosis

One of the essential roles that CIRM has played was bringing research from the bench to patients faster and empowering doctors to treat patients through clinical trials. I got into a trial and within a few months, it changed my life. CIRM met me where I was by funding a trial that gave me a future that cancer would have taken away. As a patient, I cannot express my gratitude deeply enough.

Thriving After a CIRM-Funded Stem Cell Therapy

Evangelina was expected to live out her life unable to fight off even the smallest infection, as children with ADA-SCID typically live.

ADA-SCID (Adenosine Deaminase Deficiency—Severe Combined Immunodeficiency) is a rare, inherited genetic immune disorder that prevents the body from breaking down toxins. Traditional treatment approaches use ADA enzyme therapy, or healthy bone marrow donations from a sibling that can be risky and are not always effective. Life expectancy for children with ADA-SCID was less than two years. But a CIRM-funded treatment approach from Donald Kohn, MD, at UCLA gave Evangelina a new chance at life in 2012.

Dr. Kohn extracted Evangelina's blood-forming stem cells from her bone marrow and added a normal copy of the ADA gene in the lab. From there, the modified cells were reintroduced into her body, eventually restoring her immune system.

Now Evangelina is 12 years old. She and her sister, Anabella, are competitive tennis players in Southern California. When asked about her routine checkups and blood draws, she said, "I used to cry a lot, but my mom told me that I was doing it to help and now it's fun. I know that I am helping other children.

> I don't normally stress about the points that I win or lose. I just go with the flow.

– Evangelina,
Clinical Trial Participant

Turning the Promise of Regenerative Medicine into Reality

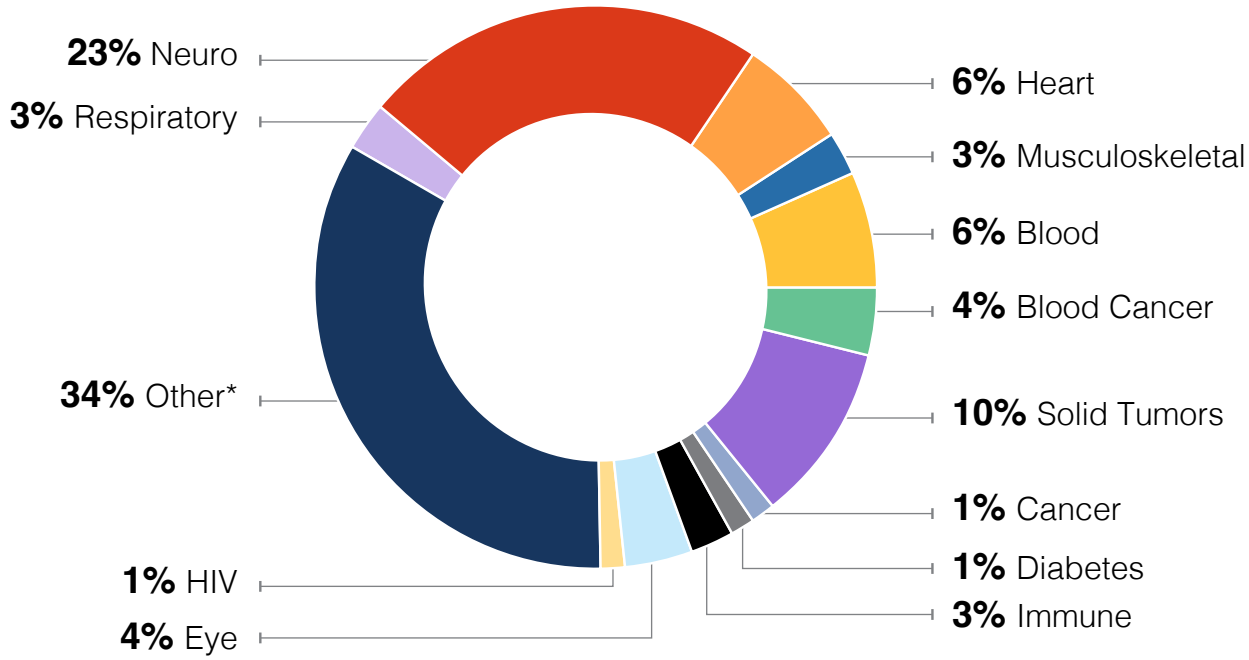
CIRM's guiding principles for the last 20 years have been innovation and discovery. The most recent evidence of this is the new CLIN4 funding program, which supports late-stage development projects by providing expert advice and accelerating outcomes along key stages of the product development pathway.

This commitment to advancing cutting-edge research has enabled CIRM to play a pivotal role in groundbreaking discoveries. CIRM has shown that developing regenerative therapies on such a scale requires technological advancement and a strategic vision.

In this section, you will learn more about how CIRM is turning the promise of regenerative medicine into reality through forward-thinking programs and initiatives.

CIRM-Funded Research Portfolio by Disease Area

Fiscal Year 2023-2024



Percentages are normalized to the total number of Research awards approved during the 2023-2024 fiscal year. Research includes discovery, translational, and clinical research awards.

Percentages rounded to the nearest whole number.

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

Photo: Jim Block courtesy of QB3

Charting the Path in Neuroscience

CIRM's Task Force on Neuroscience and Medicine was created to direct the $1.5 billion commitment to neuroscientific development as set forth in Proposition 14.

With a collective aim to develop new treatments for neurological disorders, the Task Force seeks to identify high-impact opportunities in neuropsychiatric disease, neurodegenerative disease, and neuroinjury.

Pioneering Breakthroughs Through Discovery

Neuropsychiatric disorders place a tremendous burden on patients, caregivers, and society, and represent some of the most urgent and unmet medical needs in California.

This year, CIRM launched a new initiative, called ReMIND (Research Using Multidisciplinary, Innovative Approaches in Neuro Disease), which was designed to accelerate groundbreaking discoveries in our understanding of Central Nervous System (CNS) disorders, which include diseases of the brain and the spinal cord. To achieve this, the ReMIND Program will catalyze innovative, cross-disciplinary collaborations and support broad knowledge-sharing among research scientists and other stakeholders.

The program includes two distinct funding opportunities that expedite discovery-level research in neuropsychiatric and neurodevelopmental diseases: ReMIND-L, which supports large collaborative multidisciplinary projects, and ReMIND-X, which supports exploratory, high-risk, high-reward projects.

The program will fund research at academic, non-profit, and for-profit organizations in California.

The Faces of Innovation

Elizabeth Crouch, MD, PhD, Assistant Professor, UCSF Department of Pediatrics and Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research, neonatologist physician scientist, neuroscientist, and vascular biologist was awarded a grant to explore how vascular cells develop and function in the brain. No therapies currently exist for vascular diseases of the brain that affect 12,000+ babies per year in the US.

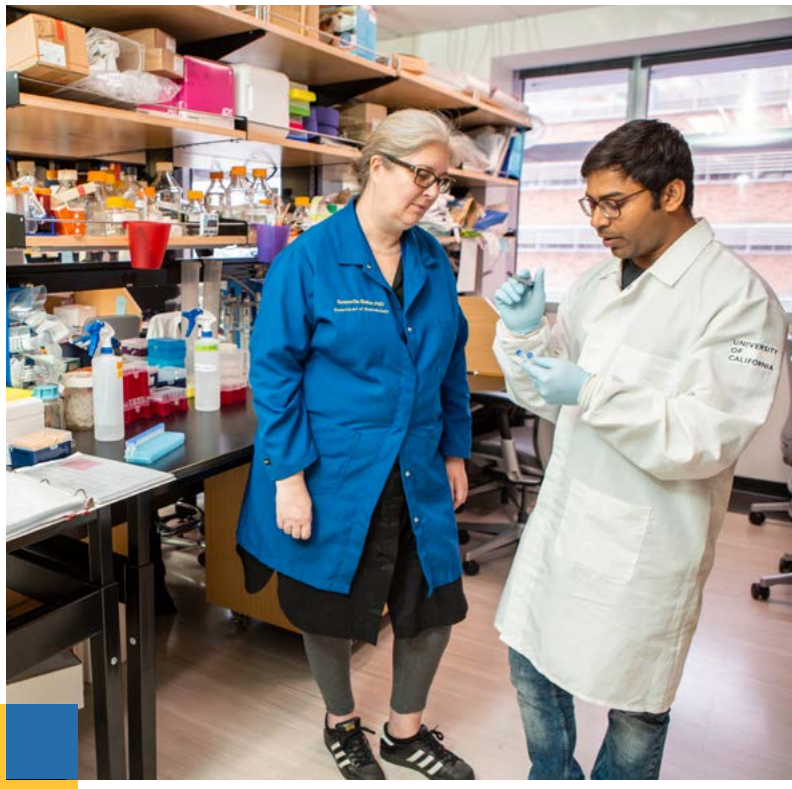
Julia Kaye, PhD, from the Gladstone Institutes, received an award to study a cellular model of sporadic ALS (amyotrophic lateral sclerosis, also known as Lou Gehrig's disease), a nervous system disease that damages the brain and spinal cord. The project will test the ability of small molecules to counteract ALS symptoms and will include the development of machine-learning technologies to detect nerve degeneration in its early stages. These advancements could significantly enhance ALS research and treatment.

Carrie Miceli, PhD, Professor in the Department of Microbiology, Immunology, and Molecular Genetics at UCLA received an award from CIRM to study the dynamics of human muscle satellite cells, myofibers, and immune cells in muscular dystrophy (MD), and test the cells' response to an experimental gene therapy. The team will have access to samples from patients with Duchenne Muscular Dystrophy who participated in a clinical trial. Insights gained from this research could improve the understanding and treatment of MD.

Michael Wilson, MD, Professor of Neurology at UCSF Weill Institute for Neurosciences, and his colleagues received an award from CIRM for an ambitious and potentially lifesaving approach to treatment for progressive multifocal leukoencephalopathy (PML) that damages nerve cells in the brain. PML is most common in people with HIV-1 but also affects those with cancer, lowered immunity, and autoimmune conditions such as multiple sclerosis, rheumatoid arthritis, and lupus. If successful, the therapy could revolutionize current treatment approaches.

Ana Moreno, PhD, Founder and CEO of Navega Therapeutics, was awarded a CIRM grant to optimize a gene therapy for inherited erythromelalgia (IEM), a rare and debilitating pain disorder. Early support from CIRM has advanced the project to further development stages. A successful outcome could significantly propel Navega's epigenetic platform, which holds promise for treating a range of conditions beyond IEM. Currently, no FDA-approved therapies exist for this disorder.

Partnerships and Collaborations Lead the Way



Integrating cutting-edge cell and gene therapies into clinical practice requires significant collaboration among researchers, clinicians, nurses, patients, advocates, and industry partners. Since its inception, CIRM has established strong partnerships with this diverse range of stakeholders, recognizing that progress is best achieved through combined efforts.

By uniting these perspectives, and fostering information sharing and diverse insights, CIRM will continue to advance regenerative medicine breakthroughs.

Photos on this page: UCLA Broad Stem Cell Research Center

Partnering for Progress: Advancing Regenerative Medicine Through Nurse Training

As regenerative therapies reach commercialization, there is a pressing need to equip nurses with more skills. Nurses play a pivotal role in the successful adoption of regenerative medicines in many settings—clinical, patient, educational, community, professional, and bedside.

In recognition of this, CIRM recently partnered with City of Hope and the Alpha Clinics Network to host the Regenerative Medicine Nurse Training Symposium.

Calling nurses “pioneers at the threshold of a new era,” CIRM Board Chair Vito Imbasciani, PhD, MD, highlighted the importance of future partnerships with nurses to accelerate the delivery of regenerative medicine in a keynote speech.

CIRM is actively listening to nurses as they lend their voices to these efforts, recognizing their essential role in this journey.

All photos on this page: Sean Richards

Supporting Bioscience Collaboration

CIRM proudly participated in and sponsored the recent Los Angeles Bioscience Ecosystem Summit Twenty 24 (LABEST) Conference, the premier showcase for bioscience innovation in Los Angeles. This conference is one of the many events CIRM supports to promote advancements in the bioscience field.

Top research programs and startups were showcased throughout the event to highlight expertise and pioneering research. The event served as a great opportunity for CIRM to highlight its impact on bioscience in the greater Los Angeles area and throughout California.

At the conference, CIRM connected with a diverse group of stakeholders, from researchers pioneering cellular therapies to entrepreneurs who turn scientific insights into therapeutic innovations. Several members of CIRM's leadership team, including Abla Creasey, PhD, Vice President of Therapeutics Development, and Shyam Patel, PhD, Senior Director of Business Development moderated panels. CIRM's President, Jonathan Thomas, PhD, JD presented a keynote, emphasizing the Agency's role in fueling innovation and its support of research across the Greater Los Angeles Area.

> **"CIRM is one of the largest public-private partnerships in the world. We have helped enable California to become a major player in the cell and gene therapy research field."**
>
> **– CIRM's President, Jonathan Thomas, PhD, JD, at the LABEST 24**

Empowering California's Research Ecosystem

As part of CIRM's commitment to overcoming obstacles in regenerative medicine, the Agency has created a $50 million funding opportunity to establish the Shared Resources Laboratories (SRLs), a network of 11 core non-profit laboratories throughout California that support the use of stem cell-based models to accelerate research and therapeutic development.

SRLs sit within CIRM's Infrastructure program which was created to address challenges to progress—in this case, broad access to stem cell-based models by researchers from diverse disciplines and backgrounds. Although there is no shortage of California laboratories and research communities interested in gaining stem cell-based modeling competency, many lack local access to laboratories, relevant infrastructure, and proper training.

The creation of SRLs delivers on Proposition 14's stipulation to re-establish a unique "Shared Labs" opportunity that first began under Proposition 71 to advance the state as a leader in the regenerative medicine space. This program also serves the goals of CIRM's strategic plan of promoting collaboration, knowledge sharing, and democratized access to technology, education, and training.

SRLs are part of several planned technology competency hubs designed to broadly connect and empower California's regenerative medicine research ecosystem.

Photo: Kellie Brown / Cal Poly Humboldt

Fostering Innovative Public-Private Partnerships

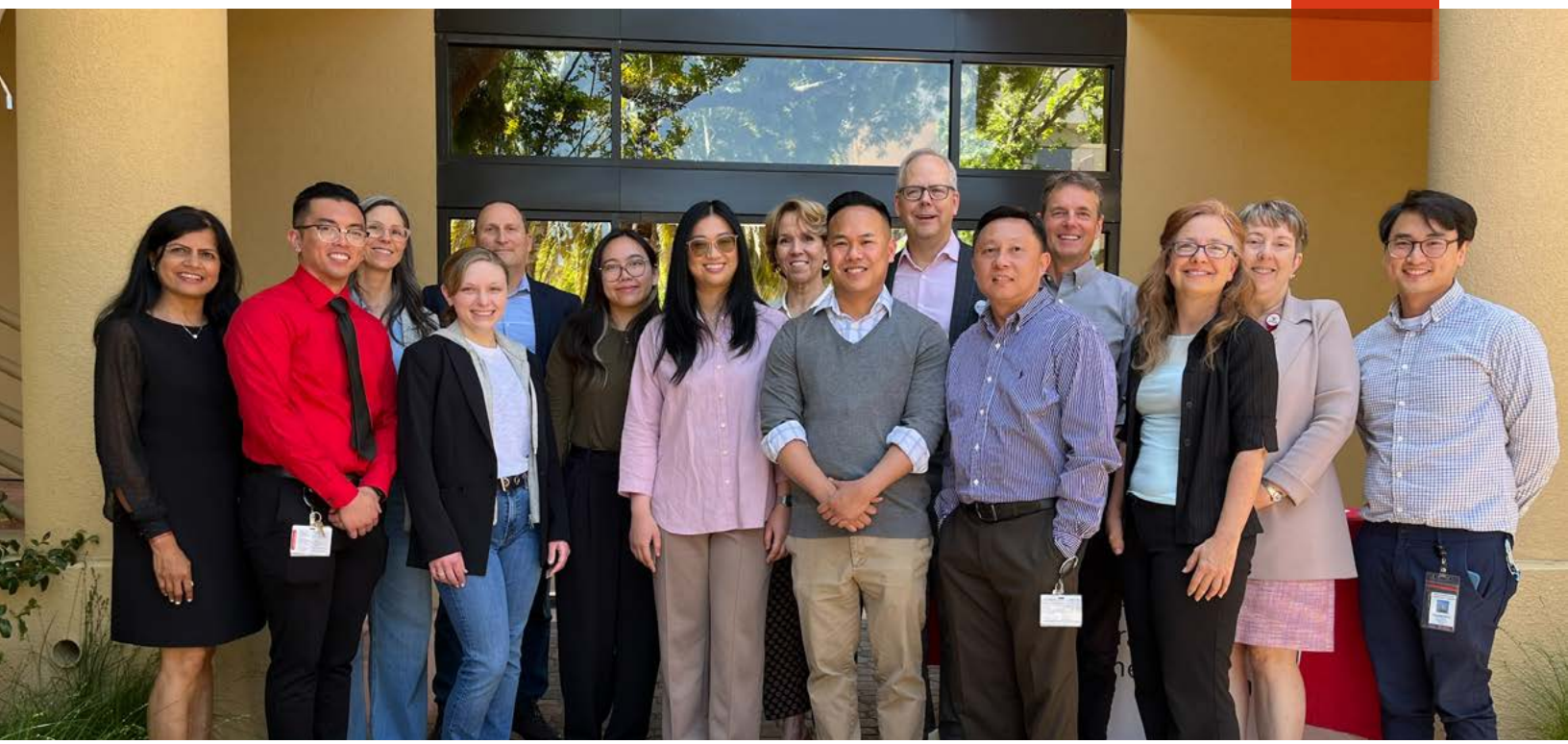
CIRM's Industry Resource Partners (IRP) is a program that facilitates collaboration between industry organizations and CIRM awardees to stimulate innovation and accelerate the development of novel therapies. The Industry Resource Partners commit to knowledge sharing and making their unique products, services, or resources readily accessible to CIRM awardees. Currently, enrolled Industry Resource Partners provide access to manufacturing services, preclinical testing, and clinical-grade stem cell lines to accelerate the development of CIRM-funded therapies. Through collaboration and knowledge sharing, the IRP advances regenerative medicine, strengthens the field, and hastens the delivery of transformative therapies to those in need.

National Resilience, Inc.

Industry Investment: A Benchmark of Success

Despite funding uncertainties and challenging market conditions, CIRM-funded projects continue to attract significant industry investments and partnerships, with $24.4 billion in follow-on funding to date. This funding has taken many forms, including co-investment, equity raises, IPOs, and acquisitions.

One of the most promising examples of industry investment came in 2024. Neurona Therapeutics successfully raised $120 million to advance NRTX-1001, a groundbreaking cell therapy for drug-resistant mesial temporal lobe epilepsy (MTLE), which was supported by an $8 million CIRM grant. The clinical-stage biotherapeutics company also received the FDA's Regenerative Medicine Advanced Therapy (RMAT) designation that speeds up the development and review of regenerative medicine therapies.



California Cell and Gene Therapy Manufacturing Network

With initial awards of nearly $18 million, CIRM announced the establishment of the California Cell and Gene Therapy Manufacturing Network, an initiative designed to overcome manufacturing bottlenecks in the development and commercialization of gene and stem cell-based therapies.

Phase 1 aims to enhance quality and operations at the nine non-profit GMP manufacturing facilities which are crucial for producing stem cell and genetic therapies for early-stage clinical trials. This phase also fosters collaboration among these facilities through knowledge sharing on technical, operational, and workforce topics. This coordination will establish best practices in manufacturing, quality control, and workforce development.

Additionally, Phase 1 develops or expands training programs to create a skilled, diverse manufacturing workforce to meet industry demand. Several awardees have launched GMP certificate programs and enrolled initial trainee cohorts with Phase 1 funding.

Phase 2 of the two-part initiative will fund collaborative academic and industry partnerships to scale operations and enhance workforce development to help accelerate the commercialization of therapies.

Photos: Stanford Laboratory for Cell and Gene Medicine (LCGM), City of Hope

City of Hope Increases Capacity with CIRM Support

A CIRM award to Taby Ahsan, PhD, of City of Hope Comprehensive Cancer Center, will enable City of Hope's on-campus manufacturing facility and her team to create platform processes and analytics for scalable manufacturing as well as analytics to enable accelerated translation of research concepts into the clinic.

"These processes are critical elements for therapeutics to advance from early clinical trials of investigational therapeutics to commercially available products."

—Taby Ahsan, PhD

CIRM Boosts Manufacturing Capabilities at UCSF

Brian Shy, MD, PhD, and his team at the University of California, San Francisco received a CIRM award to build more robust connections between various manufacturing facilities across the state. In addition to supporting CIRM-funded research, the award will enable the UCSF facility to better integrate process development and tech transfer activities, onboard next-generation platforms for cellular engineering, and establish pipelines for efficient scaling of successful products. This will help rapidly advance and translate better cell therapies for cancer, infectious disease, transplant, and rare disease indications.

"We are at the beginning of what is possible with the emerging tools and the ecosystem created by CIRM."

—Brian Shy, MD, PhD

Photos: Courtesy City of Hope and UCSF

Rocket Pharma's Gene Therapy Advances

Rocket Pharmaceuticals, a leading late-stage biotechnology company, is developing RP-L201, a lentiviral vector (LV)-based gene therapy for severe Leukocyte Adhesion Deficiency-I (LAD-I). Following compelling results from the clinical development program, which was supported by $5.9 million in funding from CIRM, the investigational gene therapy is under review with the FDA for potential approval in the US.

RP-L201 aims to deliver one-time gene therapy to patients suffering from severe LAD-I, a rare pediatric genetic immune disorder causing recurrent and fatal infections. The current treatment option, a bone marrow transplant, carries substantial risks and may not be accessible in time for affected children.

"We are grateful to CIRM for providing funding to support this critical research, as well as the patients, caregivers, and researchers who have shared this journey with us. We look forward to continuing to work towards bringing RP-L201 to patients as quickly as possible," said Kinnari Patel, PharmD, MBA, President and Chief Operating Officer, Rocket Pharma.

Top: **The Langenhops and their three children. The three siblings were born with LAD-1.**

Bottom: **Donald Kohn, MD, led the clinical trial at UCLA.**

Transforming Lives Through Gene Therapy

At nine years old, Marley Gaskins became the first patient to join a clinical trial at UCLA led by Donald Kohn, MD. The clinical trial—sponsored by Rocket Pharmaceuticals and funded by CIRM—advanced an innovative, life-changing, genetic therapy know as RP-L201 for patients with severe leukocyte adhesion deficiency-1 (LAD-I).

Marley's journey with this rare pediatric disorder that cripples the immune system began when she was a baby.

"When she was three, an abscess on her stomach landed her in the hospital for five weeks," Marley's mother, Tamara Hogue, shared.

Infections got worse and more frequent, and Marley eventually needed 24-hour attention. So, when Tamara heard about the trial, she jumped at it. "I knew in my heart this was a chance at a normal life."

Treatment involved harvesting Marley's stem cells, genetically correcting them, and injecting them back into her bloodstream to replicate. It also meant chemotherapy to clear remaining defective cells and make space for transplanted ones to grow.

Today, at age 14, Marley's body creates a continuous supply of healthy white blood cells capable of fighting infection and lets her live a typical teenage life. Because the cells are hers, there's no risk of rejection.

Doctors expect the one-time therapy will keep Marley and other LAD-I patients healthy for life.

"I knew in my heart this was a chance at a normal life."

—Tamara, Marley's mother

Photo: UC Davis Health

Above: **CuRe trial patient, Tobi, and his parents, Jeff and Michelle. Learn more about Tobi's journey on Page 29.**

Below: **Associate Director of Patient Access Geoff Lomax, DrPH and Project Manager of Patient Access Emily Crotti, MPH.**

Bringing Science and Communities Together

CIRM is committed to making regenerative medicines available to California's diverse communities, with patient access as a cornerstone of its mission. Through the Alpha Clinics Network and the future Community Care Centers of Excellence, the Agency aims to improve the availability of CIRM-funded therapies.

This effort is an ongoing collaboration between healthcare providers, community organizations, and policymakers. CIRM funding has allowed these partners to bridge the gap between research breakthroughs and patient access.

Opening Doors to New Therapies

CIRM hopes to create a more inclusive and equitable healthcare landscape in California, making groundbreaking regenerative medicine accessible to those who need it most.

The Accessibility and Affordability Working Group (AAWG) is tasked with providing recommendations to CIRM's Board, the Independent Citizens' Oversight Committee (ICOC), to examine and develop affordability strategies that enhance the accessibility of therapies resulting from CIRM-funded research. "Reflecting CIRM's commitment to access, the AAWG aims to ensure that transformative regenerative medicine is available to communities throughout California," said Maria Bonneville, Chair of the AAWG and Vice Chair of the ICOC.

This year, Chair Bonneville and ICOC Board Chair Dr. Vito Imbasciani shared CIRM's vision and key initiatives with California state legislators.

Photo: Carlos Puma

Alpha Clinics Network: Advancing Cell and Gene Therapy Through Collaboration

The CIRM-funded Alpha Clinics Network stands out as a shining example of collaborative strength. The Network is comprised of nine world-class medical centers throughout California. The Network conducts both CIRM-funded clinical trials and regenerative medicine trials on behalf of commercial and academic sponsors. These trials involve specialized doctors and nurses and incorporate novel and innovative treatment technologies.

Each center includes robust outreach programs involving partnerships with patient advocates and community stakeholders to bring visibility to clinical trials. This infrastructure is designed to meet the emerging needs of historically underrepresented communities and in collaboration with our future Community Care Centers of Excellence (CCCEs). Patients receive quality treatments from specially trained staff and the opportunity to join clinical trials—all in a single, continuum-of-care style setting. Additionally, the Network trains a workforce to sustain these efforts over time.

The key to the Network's success is its ability to accelerate progress through collaboration, knowledge sharing, and the capacity to deliver complex treatments in an efficient and effective manner.

Making an Impact

40+ Disease Areas

250+ Clinical Trials

2,000+ Clinical Trial Participants

Nine Leading Medical Centers

Cedars-Sinai Medical Center

City of Hope

Stanford University

University of California, Davis

University of California, Irvine

University of California, Los Angeles

University of California, San Diego

University of California, San Francisco

University of Southern California/ Children's Hospital Los Angeles

Community Care Centers of Excellence: Increasing Access to Clinical Trials throughout California

The California Stem Cell Research, Treatments, and Cures Initiative of 2020 (Proposition 14) mandates the establishment of Community Care Centers of Excellence (CCCEs) to promote access to programs arising from CIRM-funded research. CCCEs fall under CIRM's Infrastructure program and serve its strategic vision to deliver real-world solutions, expand workforce career development opportunities, and promote inclusivity and diversity in the field.

In January 2024, CIRM approved a $60 million funding opportunity for the CCCEs. Through this new initiative, CIRM aims to establish geographically diverse and culturally responsive centers of excellence to support access to clinical trials or approved regenerative medicine treatments.

Photo: Barbara Ries for UCSF

Above: **Jennifer M. Puck, MD dances with HT, a young patient who participated in a CIRM-funded clinical trial at UCSF for Artemis-SCID, a rare genetic disorder.** ***Below:*** **HT and his family in the Navajo Nation**

Photo: Gillian Grisman / UCSF

Breaking Down Barriers to Clinical Trials

The opportunity for patients to participate in clinical trials can be lifesaving but also very challenging. Financial hardships, long-distance travel, and family commitments can prevent many people from getting to and staying in clinical trials.

Recognizing these barriers, CIRM is committed to supporting patients through the clinical trial process via the Patient Support Program. Funds for the Patient Support Program are set aside under Proposition 14 to address the informational, financial, and logistical bottlenecks experienced by clinical trial patients and their families.

As part of this commitment, the Patient Support Program will offer a range of services, including referrals to clinical trials and trial navigation. By reducing costs and enhancing representation in trials, this initiative promotes a more inclusive and equitable healthcare landscape, furthering CIRM's mission.

Pediatric allergy and immunology specialist Caroline Y. Kuo, MD with a patient at the UCLA Broad Stem Cell Research Center, a CIRM-funded institution.

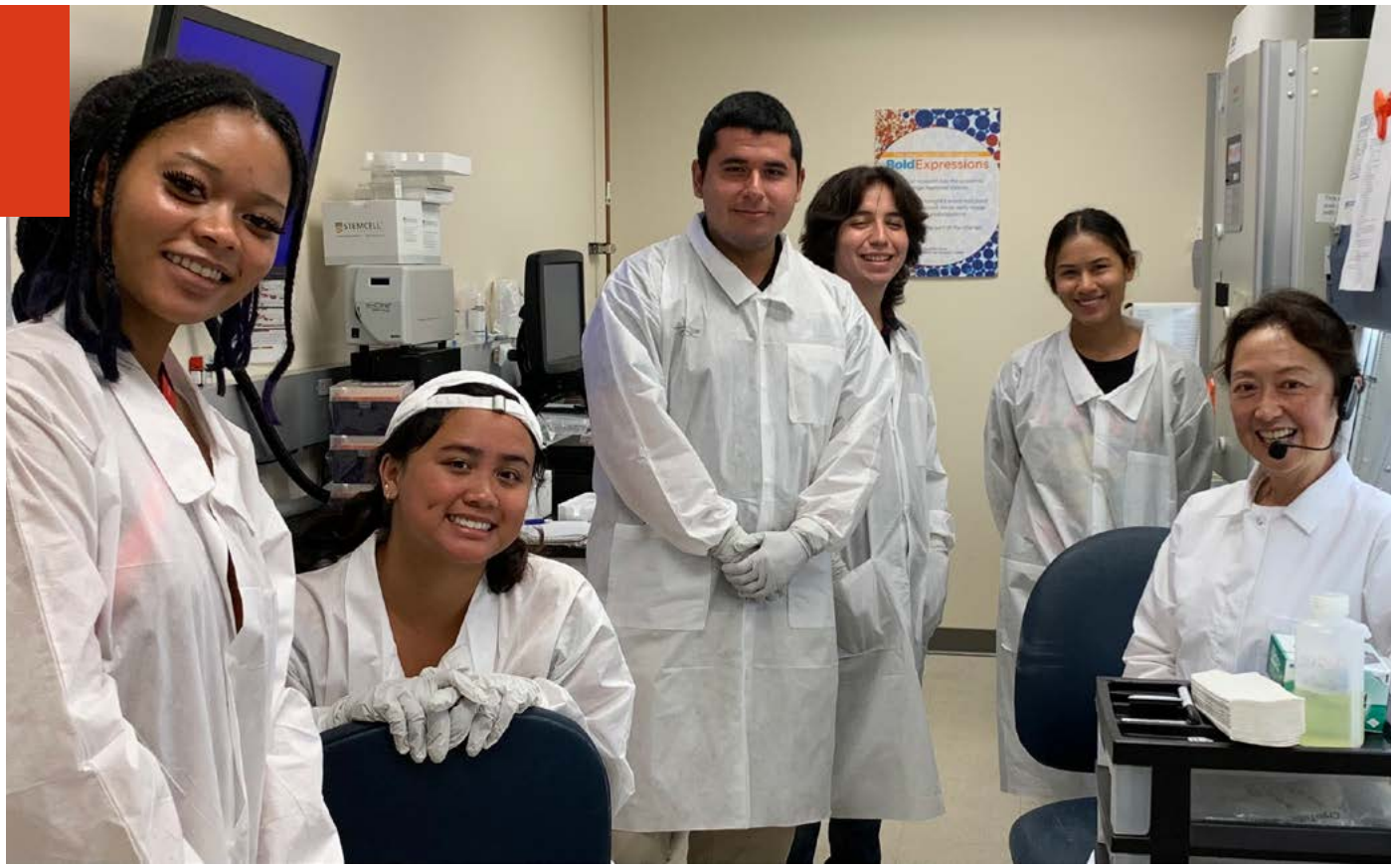
The CIRM team spreads awareness of sickle cell disease and stem cells at the 2024 Black Joy Parade in Oakland, CA.

Celebrating Power and Community

As part of ongoing community outreach efforts led by the communications and outreach team, CIRM proudly participated in the 2024 Black Joy Parade. This yearly celebration of the Black community began with a spirited parade in Oakland and culminated in a celebration featuring local businesses, community-based organizations, and musical artists.

The focus of this year's event was primarily on celebrating joy, yet it also highlighted another important aspect: CIRM's commitment to funding regenerative medicine research for Sickle Cell Disease (SCD), which affects approximately 100,000 individuals in the United States. To date, CIRM has allocated over $65 million towards research, manufacturing, and development of SCD therapies.

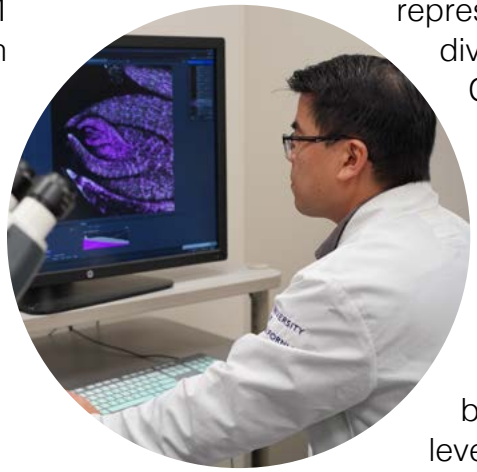
This engagement is a prime example of CIRM's ongoing outreach efforts. These events are designed to engage with communities, provide education on regenerative medicine, and cultivate strategic partnerships.



DEI: Driving Growth, Championing Change

Diversity, equity, and inclusion (DEI) are integral to every program CIRM supports. The Agency believes all people deserve equal access to the benefits of regenerative medicine.

To further this commitment, CIRM requires diverse representation in the projects it funds. Proposals must reflect an understanding of health disparities associated with the target indication and be accompanied by an action plan that demonstrates proactive engagement with diverse populations and strategies to foster cultural sensitivity.



Requiring that clinical trials expand representation is a major step forward in reversing structural healthcare inequity.

Another goal of CIRM is to bolster a diverse and skilled workforce representative of California's diverse population. CIRM provides educational and training opportunities in regenerative medicine for students and young professionals with varied educational backgrounds and skill levels.

Photos: Courtesy UC Irvine COMPASS Scholarship Program, UCLA Broad Stem Cell Research Center

Advancing a Stem Cell Treatment for Spina Bifida

This past year, CIRM awarded a $9 million grant to Diana Farmer, MD at UC Davis Health and chief of pediatric surgery at Shriners Children's Northern California to support the completion of a Phase 2 clinical trial to improve the outcomes for children born with a severe form of spina bifida called myelomeningocele.

Spina bifida is a birth defect that occurs when the spine and spinal cord aren't formed properly during fetal development, usually resulting in life-long lower body paralysis.

The CIRM award will support the advancement of a one-of-a-kind treatment delivered while a fetus is still developing in the mother's womb via a patch coated with placenta-derived stem cells. The treatment is meant to prevent paralysis and preserve normal bodily function, and ultimately improve the quality of life of these affected children and their families.

Michelle Johnson and Jeff Maginnis agreed to participate in the Phase 1 trial at UC Davis Health when they discovered via an ultrasound exam at 20 weeks into the pregnancy that the developing fetus had spina bifida.

Months after receiving the treatment in the womb, Michelle gave birth to a healthy baby boy named Tobi, who early on, began hitting all his milestones and wriggling his legs.

Today, Tobi is "doing excellent" and able to walk independently, his mom Michelle says. "He loves to swim, climb, jump on the trampoline, and ride his green tractor."

Photos: Courtesy Michelle Johnson

Photos by Hartlove-Goodyear

Carrying Our Vision Forward

CIRM's Education and Training programs are a vital link to tomorrow.

CIRM offers traditionally underserved students various opportunities to participate in training programs. These initiatives prepare a new generation of scientists for cell and gene therapies emerging from today's field.

By establishing a workforce of highly skilled and diverse scientists, CIRM is ensuring that its impact on research and development, infrastructure, community and patient outreach, and DEI initiatives continue into the future.

Training the Next Generation of Scientists

CIRM offers valuable educational and training pathways in regenerative medicine tailored for students and young professionals from California's diverse communities.

Four CIRM-funded programs—SPARK, Bridges, COMPASS, and Scholars—support the next generation of regenerative medicine scientists from high school to clinical fellows. Each program offers hands-on experience that builds towards mastery of the technical skills critical to future scientific and medical advancement.

CIRM's Educational Vision of the Future

This year, CIRM launched its education network strategy, representing a significant step in the Agency's commitment to education. These initiatives seamlessly integrate educational programs with practical applications, connecting training to real-world use and leveraging investments in CIRM's Education and Infrastructure pillar programs.

Education and Training Programs: New and Diverse Perspectives at the Scientific Table

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.

Faith "Andy" Anderson

Charles R. Drew University of Medicine and Science

SPARK Trainee 2023

"This field excites me because it has so much potential. My favorite part of the SPARK conference was seeing women and people of color who look like me doing groundbreaking work."

BRIDGES

Bridges to Stem Cell Research and Therapy

15 PROGRAMS STATEWIDE

Bridges prepares diverse and disadvantaged candidates in undergraduate and master's degrees in science, for research and career opportunities in regenerative medicine.

David Mendoza Bautista

Master's Student Intern, Novo Nordisk

Cal Poly Bridges Trainee 2023-2024

"The Bridges program is really about personal growth and collective innovation."

COMPASS

Creating Opportunities Through Mentorship and Partnership Across Stem Cell Science

16 PROGRAMS STATEWIDE

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

Ryan Johnson

Undergraduate Student

UC Riverside COMPASS Trainee 2023-24

"The best part has been all the hands-on work, and just being a part of regenerative medicine as a whole."

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 fellows. Scholars contribute to the expansion of stem cell research as they become skilled researchers and aspiring regenerative medicine leaders.

Orchid Garcia

Postdoctoral Fellowship, 2013-2014

Children's Hospital Los Angeles - The Saban Research Institute

"Most inspiring is the diversity of thought that the program encompasses to tackle some of the most complex clinical and scientific challenges."

Photo courtesy of Orchid Garcia

Budget Update

CIRM's mission and strategy are anchored in five critical areas of investment: Discovery, Translational, Clinical, Education, and Infrastructure. Each pillar is essential for advancing stem cell and gene therapy research at every stage.

Budget for Fiscal Year 2023-24: $519 million

Research Budget for Fiscal Year 2023-24	$	519 million
Administrative Budget for Fiscal Year 2023-2024	$	28.9 million
Funds Approved (grants and awards)	$	377.4 million
Funds Remaining	$	141.5 million
Balance Under Active Management	$	627.5 million
Number of Active Project Awards		342

Approved Six Month Budget for Fiscal Year 2024-25*: $420.5 million

Discovery Research

$116.2 million

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

Translational Research

$60 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

$145.5 million

CIRM is 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

$0**

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

Infrastructure Program

$88.8 million

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

**Supports new awards from July—December 2024. CIRM will request funds for the remainder of the fiscal year in December 2024.*

***Current education programs are fully funded for the current fiscal year.*

People Catalyze the Path Forward

At CIRM, the pursuit of positive change and the commitment to pioneering advancements in regenerative medicine are fundamental principles. These forces motivate the CIRM team to work hard to achieve what once seemed unattainable.

CIRM acknowledges that it truly takes a village to succeed. The Agency understands that the most significant scientific advancements emerge from a community rich in diverse perspectives.

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20TH ANNIVERSARY

2024 ANNUAL REPORT

OUR JOURNEY FORWARD: HOW CIRM CATALYZES REGENERATIVE MEDICINE TODAY AND BEYOND