

CIRM

CALIFORNIA INSTITUTE FOR
REGENERATIVE MEDICINE

2025 ANNUAL REPORT



LOOKING FORWARD

ADVANCING CALIFORNIA'S
SCIENTIFIC VISION



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.

Over 20 years ago, California voters made history by passing Proposition 71, creating the California Institute for Regenerative Medicine (CIRM): the first state agency dedicated to funding stem cell research and advancing regenerative medicine.

This bold approval and investment by voters launched CIRM's mission to accelerate the development of transformative therapies and, in doing so, positioned California as a global leader in regenerative medicine. In 2020, voters reaffirmed their commitment by passing Proposition 14, providing \$5.5 billion in renewed

funding to continue addressing diseases with urgent, unmet medical needs.

Since its inception, CIRM has fast-tracked the development of innovative treatments while helping build a thriving biotech ecosystem across the state including research labs, clinics, manufacturing facilities, training programs, and strategic industry partnerships. These efforts have accelerated the timeline from scientific discovery to real-world impact for patients.

As CIRM enters its third decade, its commitment endures: to accelerate regenerative medicine for the health and well-being of Californians.

Photo credits: The Adler Lab—UC San Diego, Cedars-Sinai

Contents

- 02** Leadership Letters
- 03** Our Impact
- 04** Strategic Goals
- 06** Accelerating Discovery & Translation
- 12** Cell & Gene Therapy Approvals
- 20** Accessibility & Affordability
- 26** Workforce Development
- 33** Budget Snapshot



The Power of Regenerative Medicine

Regenerative medicine has entered a new era. Once considered a future endeavor, therapies involving stem cells, gene editing, and other advanced biotechnologies are now transforming how we treat serious diseases. These cutting-edge approaches are redefining what's possible for conditions like cancer, diabetes, Parkinson's disease, genetic disorders, and many others.

CIRM continues to play a critical role in advancing this progress by funding world-class research and accelerating the translation of scientific breakthroughs into potential real-world treatments.

But the work goes beyond developing new therapies—it's about revolutionizing science and building the infrastructure needed to deliver treatments.

Through its education and training initiatives, CIRM is also cultivating the next generation of scientific leaders. These efforts help Californians access regenerative medicine careers and support workforce development to keep the state's regenerative medicine industry strong.

In conjunction with researchers, clinicians, industry, and patient advocates across California and the world, CIRM remains committed to delivering on its goal of advancing regenerative medicine science to improve lives.

Dear Fellow Californians,

As we look toward the future, CIRM remains committed to advancing groundbreaking innovation and improving the lives of Californians and people around the world through regenerative medicine.

This year's annual report theme—Looking Forward: Advancing California's Scientific Vision—reflects the dynamic and ever-evolving landscape of scientific research and our pivotal role in shaping it.

This past year, our Board approved new impact goals as part of our Strategic Allocation Framework in an effort to ensure CIRM-funded research and initiatives have maximum impact on the people of California. This framework will advance our mission in many transformative ways by streamlining the research-to-treatment pipeline, focusing on high-impact diseases, and expanding access to regenerative medicine, ultimately driving more therapies closer to patients who need them.

In 2025, we are not only celebrating the progress we have made, but more importantly, we are accelerating toward the next horizon of possibility. The journey ahead is both exciting and full of promise, and we are honored to lead these efforts with your support.

Sincerely,



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

**Dear Fellow Californians,**

This past year marked a pivotal transition for both me and CIRM. After 12 years as Chair of the Board, I stepped into the role of full-time President and CEO—an honor that allows me to work closely with our exceptional team to further our goal of delivering transformative therapies to patients with unmet medical needs.

In 2024, we conducted a rigorous, data-driven analysis of disease burden across California, leading to new strategic impact goals and a roadmap for deploying the remainder of our Proposition 14 funds. This includes launching updated funding programs across multiple research pillars, ensuring continued support for California scientists, especially amid ongoing federal funding uncertainty.

At CIRM, we are meeting today's challenges by cultivating a research ecosystem where innovation thrives, and bold ideas move toward clinical impact. Each step forward brings us closer to life-changing regenerative medicine treatments and advances the scientific vision California voters entrusted to us more than 20 years ago.

As we look forward, we remain energized by the lasting impact our work will have across the state and beyond.

Sincerely,



Jonathan Thomas, PhD, JD
President and CEO





Building a Scientific Ecosystem in California

For more than 20 years, CIRM has played a pivotal role in cultivating a robust regenerative medicine ecosystem across the state. Through strategic investments and collaborative partnerships, CIRM has empowered researchers, accelerated the development of cell and gene therapies, and strengthened the infrastructure of California's research institutions. Here are a few ways that CIRM programs have benefitted people in California, based on data as of June 30, 2025.

\$4.6 Billion

Total funds approved to support cell and gene therapy research, education, and infrastructure in California

85+ Diseases/ Conditions

CIRM funding supports research projects to address a wide range of health issues, from arthritis to Alzheimer's disease, cancer, rare diseases, heart disease, and stroke

56,000+ Full-time Equivalent Jobs*

Additional full-time equivalent jobs created in California (*Economic impact under Proposition 71)

4,300+ Participants in Clinical Trials

Patients who have enrolled in clinical trials supported by CIRM

4,600+ Interns and Fellows

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

116 CIRM-Funded Clinical Trials

By directing support for 116 clinical trials, CIRM plays a crucial role in advancing its mission to deliver innovative stem cell and gene therapies to patients

Charting the Future: CIRM's Strategic Goals

In 2024, CIRM launched its Strategic Allocation Framework, a data-driven initiative to guide the investment of the agency's remaining \$3.86 billion, including \$1.14 billion dedicated to neurological research.

Our framework outlines six goals (see next page) across four guiding principles: Accelerating Discovery & Translation, Cell & Gene Therapy Approvals, Accessibility & Affordability, and Workforce Development. These priorities are designed to maximize impact by aligning funding with measurable goals.

The process of creating this plan leveraged internal data, external research, and stakeholder input to identify funding areas with the greatest potential for impact. This effort builds on CIRM's

collaborative tradition, with further input from key committees leading to formal recommendations to the Board in September 2024. It reflects CIRM's ongoing commitment to shaping strategic directions in close partnership with the Board.

"CIRM has made a significant impact on the research ecosystem in California. Our new priorities will refine that process, making sure that every dollar is strategically allocated through impact goals and data-driven analysis," said Rosa Canet-Avilés, PhD, CIRM Chief Science Officer (CSO), who spearheaded the effort.

With this framework, CIRM is poised to lead the next era of regenerative medicine by funding smarter, translating research faster, and ensuring our impact reaches further.



Focused, Strategic, & Impactful

The following are goals within each key category from CIRM's Strategic Allocation Framework:

Accelerating Discovery & Translation

GOAL 1: Catalyze the identification and validation of at least four novel targets and biomarkers, ensuring integration into preclinical or clinical research for diseases in California

GOAL 2: Accelerate the development and utilization of 5-8 technologies that have the potential to improve safety, efficacy, and/or quality of cell and gene therapies

Cell & Gene Therapy Approvals

GOAL 3: Advance 4-7 rare disease projects to Biologics License Application (BLA)

GOAL 4: Propel 15-20 therapies targeting diseases affecting Californians to late-stage trials

Accessibility & Affordability

GOAL 5: Ensure that every BLA-ready program has a strategy for access and affordability

Workforce Development

GOAL 6: Bolster CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine

Learn more about strategic goals at cirm.ca.gov/strategic-goals.

Fueling Bold Science

CIRM is accelerating the development of stem cell and gene therapies by investing in bold science and building the infrastructure to move discoveries toward clinical impact.

In 2024, we expanded our support for early-stage research through new Discovery programs that identify novel targets and biomarkers for potential therapies, foster multidisciplinary collaboration, and engage industry perspectives early to ensure innovations can become scalable and clinically relevant.

To further accelerate progress, CIRM launched new funding programs this past year—including DISC4 and DISC5—to support comprehensive discovery research to encourage collaborative,

multidisciplinary innovation in stem cell and genetic research.

CIRM also introduced new data initiatives to improve how researchers collect, integrate, and share information across disease areas—empowering collaboration and advancing data-driven discovery. In addition, CIRM is advancing technologies that enhance the safety, efficacy, and quality of cell and gene therapies through future incubation programs that unite academic and industry partners, helping move tools swiftly toward clinical and commercial readiness.

Together, these efforts are fast-tracking regenerative medicine breakthroughs for patients in need.

Photo credit: Elena Zhukova—UCLA Broad Stem Cell Research Center

Photo credits: Alex Pollen ReMIND Project, Steve Babuljak

Advancing Collaborative Neuroscience Research



Neuropsychiatric and neurodevelopmental disorders are among the most urgent medical needs in California, placing a heavy burden on individuals, families, and communities. In 2024, CIRM launched the ReMIND Initiative (Research using Multidisciplinary, Innovative Approaches in Neuro Diseases) to advance our understanding of central nervous system disorders and lay the foundation for future research.

Through this program—which has since served as a model for our new Discovery (DISC4) funding program—CIRM committed over \$84 million to fund seven large-scale, collaborative projects across ten leading California institutions. These teams are investigating the cellular and

molecular mechanisms underlying complex conditions such as autism, schizophrenia, and intellectual disability.

Together, the seven teams form a network of 44 investigators who are applying cutting-edge tools including brain organoids, gene editing, proteomics, metabolomics, and machine learning approaches to address critical questions in brain development and disease.

Awardees engage in structured knowledge-sharing throughout the four-year award period, with CIRM facilitating integration and partnerships across teams. This coordinated model strengthens opportunities for shared discovery and accelerates the path to clinical translation.



▶ *Alex Pollen, PhD, Associate Professor of Neurology at UC San Francisco, is leading a \$12.2 million project with investigators at UC Berkeley and UC Santa Cruz to study the genetic foundations of autism spectrum disorder. Using patient-derived brain organoids (3D cell cultures derived from stem cells), the team aims to identify biomarkers and genetic modifiers that influence disease severity.*

Advancing Early-Stage Research

At CIRM, we recognize that the journey from scientific discovery to clinical application requires a robust foundation. Our Discovery and Translational (now known as Preclinical Development) funding programs are designed to bridge this gap by supporting early-stage investigations and the preclinical development of stem cell-based and genetic therapies. Through these programs, we empower researchers to explore novel technologies and advance them toward clinical use.

Photo credits: Elena Zhukova—UCLA Broad Stem Cell Research Center

Below is a snapshot of Discovery and Translational awards made by CIRM over the past fiscal year.

Xin Jin, PhD, of Scripps Research, and the Howard Hughes Medical Institute (HHMI), is studying how autism risk genes affect brain development by applying scalable genetic screening tools and spatially resolved measurements to 3D stem cell-derived models. These approaches allow researchers to interrogate how these genes impact individual cells during brain formation and maturation. By uncovering how these genes influence brain development, this research could lead to new ways to understand, prevent, or treat autism in the future.



Robert Negrin, MD, of Stanford University, is developing a way of controlling the excessive and dysregulated immune response following a stem cell or organ transplant. The approach involves modifying a type of immune cell called a regulatory T cell to have a molecular switch that allows activation and expansion of these cells in the body.



Jennifer Johnston, PhD, of NysnoBio GT Neurology, is advancing a therapy to treat Parkinson's disease caused by mutations in a gene called Parkin. People with Parkin mutations often develop Parkinson's disease before the age of 45. The therapy will restore the Parkin gene, regenerate the dopamine neurons, and mitigate disease progression. This approach can also be tested in sporadic Parkinson's disease once clinical benefit has been demonstrated in the genetic population.



Sewoon Han, PhD, of CellFE, Inc., is working on a faster, more efficient way to make regenerative cell therapies. His team is developing a device and process that can significantly shorten the gene-editing process compared to current methods. This faster approach helps keep the cells in their most powerful, stem-like state, which can make treatments more effective. If successful, this technology could lead to better, more reliable therapies for diseases like cancer and blood disorders, and make it easier to produce these treatments on a large scale.



“Currently, a lot of data has been published, hundreds of datasets on the cerebral cortex alone, but it takes hours to convert the datasets and look at them. Our websites will reduce this to seconds”.

– Maximilian Haeussler, PhD



Microscopy image of a brain organoid contributed by CIRM awardee Aparna Bhaduri, PhD.

Powering Science with Shared Data

Sharing research data accelerates scientific discovery, enhances reproducibility, and fosters collaboration by allowing other researchers to validate findings and build on existing work. CIRM supports this open science approach by requiring that CIRM-funded research data be shared in ways that are accessible, transparent, and respectful of patient privacy.

To make data more discoverable and usable, CIRM developed the Data Explorer (datahub.cirm.ca.gov), a centralized dashboard that compiles information on more than 900 experiments funded throughout CIRM's history. This platform helps researchers locate and reuse valuable datasets, maximizing the impact of CIRM's investment in regenerative medicine research.

CIRM also provides funding for innovative tools that promote data sharing. For example, Maximilian Haeussler, PhD, of UC Santa Cruz is creating a virtual microscope to explore gene expression in brain organoids, which are 3D cell cultures derived from stem cells that model brain development and brain disorders. These complex datasets are challenging to analyze, but tools like the Cell Browser (cells.ucsc.edu) allow scientists to see which genes are active or inactive and even upload and explore their own data.

By investing in infrastructure and technology for open data, CIRM helps researchers worldwide advance regenerative medicine more efficiently and collaboratively.

Shared Resources Laboratories: Advancing Science Statewide

CIRM's Shared Resources Laboratories for Stem Cell-Based Modeling play a vital role in strengthening California's regenerative medicine ecosystem. Across the state, these specialized labs provide researchers—regardless of institutional affiliation—with access to cutting-edge technologies, stem cell lines, training, and expert support. By lowering barriers to complex stem cell-based discovery research, these labs foster innovation, collaboration, and scientific rigor.

CIRM has awarded more than \$48 million to support a network of 11 such labs, an important component of CIRM's Infrastructure Program designed to address challenges that hinder progress in the regenerative medicine field.

Supporting researchers and educators across California to use stem cells to model human biology and disease, these labs help lay the groundwork for discovering and developing new treatments, demonstrating CIRM's commitment to innovation and long-term impact for Californians.

More than \$48 million in CIRM funding supports a network of 11 Shared Resources Laboratories across various institutions, including:

Northern California

Cal Poly Humboldt
Gladstone Institutes

Central California

University of California, Merced

Southern California

Cedars-Sinai
City of Hope
The Lundquist Institute
The Salk Institute
University of California, Irvine
University of California, Los Angeles
University of California, Santa Barbara
University of Southern California



◀ *The UCLA-CIRM Shared Resources Lab, led by Kathrin Plath, PhD, is providing access to a flexible, automated method for stem cell modeling that boosts speed, consistency, and accessibility. It offers users access to human stem cell lines, gene modification tools, and support in differentiating stem cells into various cell types, speeding research and therapy development for scientists.*

Bringing Cell and Gene Therapies to Patients

Cell and gene therapies hold the potential to transform how we treat a wide range of diseases, from rare genetic conditions to common chronic illnesses. For this potential to be realized, these therapies must move beyond initial research in labs to carefully designed clinical trials where they are tested in human patients for safety and efficacy.

Securing U.S. Food and Drug Administration (FDA) approval for a therapy is a critical milestone that validates a therapy's safety and effectiveness, opening the door for broader access and real-world use.

By funding early research, supporting rigorous preclinical projects and clinical trials, and fostering collaborations between academic researchers and industry, CIRM helps advance therapies through every stage of development.

CIRM-funded programs continue to advance toward key FDA milestones, with several therapies entering first-in-human trials, late-stage trials or attracting major biopharma investment. In the past fiscal year, nine projects were submitted for FDA clearance to begin human trials: a process called an Investigational New Drug (IND) application. Six of these CIRM-supported projects received clearance to move forward.

These successes reflect CIRM's strategic investments and long-term commitment to building a pipeline of therapies aimed at delivering lasting impact for patients.

To help meet therapy approval goals, CIRM has launched new and updated funding opportunities, including Preclinical Development (PDEV) and Clinical Trial (CLIN2) programs. The programs will approve new awards in early 2026.

CIRM-Supported Epilepsy Trial Offers Hope for New Treatment

In 2007, Justin Graves (pictured), a passionate SCUBA diver, was diagnosed with epilepsy, a condition that not only forced him to stop diving but also made him unable to drive. Despite trying several medications, his seizures persisted, affecting his quality of life.

In 2023, Justin enrolled in a clinical trial led by Neurona Therapeutics, a South San Francisco biotech company that had received \$18 million in funding from CIRM to develop a stem cell-based therapy for unilateral epilepsy, which originates in one side of the brain.

The trial tested NRTX-1001, a potential treatment in which inhibitory neurons derived from human stem cells are injected into the brain to help reduce seizures. Justin, who had been struggling with seizures for years, began noticing improvements within a month of receiving the therapy. While he still experiences occasional minor seizures called auras, he no longer suffers from more severe or grand mal seizures.

Like Justin, other patients who were treated in this clinical trial had substantial and durable improvements

in the frequency and severity of seizures that they have suffered, and the therapy has been well tolerated. The success to date of this ongoing Phase 1/2 trial offers hope to patients and families for the advancement of a regenerative cell therapy for drug-resistant epilepsy, representing a potential alternative to traditional surgical lobectomy approaches that can cause severe and irreversible side effects.

In March 2025, CIRM approved a new \$14 million award to help fund Neurona's Phase 1/2 clinical trial of NRTX-1001 in drug-resistant bilateral epilepsy, which affects both sides of the brain.

Neurona is also planning a Phase 3 trial to further assess the therapy's effectiveness, with hopes of FDA approval. Last year, Neurona received the FDA's Regenerative Medicine Advanced Therapy (RMAT) designation for NRTX-1001, which now enables additional dialogue with the FDA to explore potential ways to accelerate clinical development.

Justin is proud to be part of this pioneering work. "It's a huge step forward," he said, "not just for me, but for everyone."



Uniting Experts to Address Manufacturing Challenges

This past year, CIRM hosted its inaugural Manufacturing Network Symposium in South San Francisco, bringing together more than 100 leaders in the cell and gene therapy industry to tackle shared manufacturing challenges. Sponsored by Forge Biologics and Avid Bioservices, the event featured academic and industry experts emphasizing the importance of collaboration, standardization, and patient-centered innovation.

Speakers from various institutions shared case studies and insights on overcoming barriers like infrastructure limitations, lengthy contracting, and intellectual property concerns. Participants stressed the need to move beyond competition and streamline efforts to expand access to promising therapies, as well as the importance of cross-sector partnerships to improve cell and gene therapy scalability and accessibility.

In support of these manufacturing efforts, CIRM has invested \$18 million to launch and support a Manufacturing Network, uniting nine academic Good Manufacturing Practices facilities to address production bottlenecks and elevate manufacturing capabilities statewide.





▶ This year, the Cedars-Sinai Biomanufacturing Center—part of the CIRM Manufacturing Network—celebrated its second Biomanufacturing Internship Program cohort with a showcase event.

Over 14 weeks, interns chosen from a competitive applicant pool gained hands-on experience in identifying types of stem cells, transitioning those cells into other types of mature cells, and gene editing.

Supported by CIRM funding, the program offered early-career scientists the chance to work with and be mentored by experts on cutting-edge research. The showcase highlighted their contributions and underscored the program's role in training the next generation of biomanufacturing leaders.



Driving Research for Vision Restoration

In California, more than 700,000 people have a form of vision loss or impairment. Worldwide, the number reaches into the millions.

To help combat this issue, CIRM is advancing cell and gene therapies for vision loss by supporting more than 60 projects and awarding over \$200 million in funding to date. These efforts focus on major causes of blindness, including age-related macular degeneration (AMD) and retinitis pigmentosa.

Recently, CIRM awarded nearly \$8 million to Paul Bresge of Ray Therapeutics to launch a Phase 1 clinical trial for a novel gene therapy for retinitis pigmentosa, building on earlier work supported by a CIRM Translational grant. CIRM also awarded \$12 million to Jane Lebkowski, PhD, of Regenerative Patch Technologies for a Phase 2b trial of a stem cell-based retinal implant for a severe form of AMD.

Meanwhile, Jeffery Stern, PhD, MD, of Luxa Biotechnology—who is leading a Phase 1/2a

clinical trial funded in part by a \$4 million CIRM grant—earned a key regulatory milestone with FDA’s Regenerative Medicine Advanced Therapy (RMAT) designation for its dry AMD therapy made from adult retinal pigment epithelial stem cells. This designation can help the company expedite development of the treatment by working more closely with the FDA throughout the process.

In another important achievement, CIRM-funded research at UCLA led by Anthony Aldave, MD received both Orphan Drug and Rare Pediatric Disease designations for a gene therapy targeting congenital hereditary endothelial dystrophy, a genetic eye disorder that affects the cornea and leads to vision problems.

These milestones and awards underscore the quality of the work that CIRM funds in its efforts to bring life-changing treatments to people affected by vision loss.

Seeing the Light: One Patient's Stem Cell Journey

Kristin MacDonald was the first patient enrolled in a clinical trial funded by CIRM and led by Newport Beach-based biotech company jCyte for retinitis pigmentosa, a degenerative eye disease she was diagnosed with in her late 20s after years of progressive vision loss. Once an aspiring actress and talk show host, Kristin described her diagnosis as a gradual and difficult reality that reshaped her life.

Eight years ago, she received her first stem cell injection as part of the trial. It was a moment she remembers vividly, from the anticipation in the room to the careful coordination among the research team. Two months later, she began noticing a bright light in her vision. That change has lasted ever since and continues to help her navigate daily activities like swimming and going to the gym.

Since participating in the trial, Kristin has remained a dedicated advocate for regenerative medicine and for others affected by vision loss. As the founder of Second Vision and a public speaker, she continues to support CIRM's mission by sharing her story and helping raise awareness about the potential of stem cell therapies.



Kristin MacDonald shares her experience as the first participant in a CIRM-funded clinical trial for retinitis pigmentosa, a journey that brought lasting improvements to her vision and quality of life.

Advancing Therapies Through Partnerships

CIRM's Industry Resource Partners (IRP) Program advances CIRM's strategic goals by fostering strategic partnerships between biotech companies and CIRM-funded researchers to accelerate the development and commercialization of cell and gene therapies.

By addressing bottlenecks in the research pipeline—particularly around manufacturing, regulatory strategy, and technology access—the program aims to move projects from discovery stage research through clinical development. Participating companies agree to contribute critical services such as project consultation, access to gene editing tools, scalable manufacturing, and regulatory

guidance. As of June 2025, CIRM's IRP Program consists of 24 members offering paid services to CIRM-funded researchers.

Through shared resources, mentorship, and engagement in CIRM symposia and advisory panels, program members provided expertise to a network of over 200 CIRM-funded cell and gene therapy projects.

Aligned with CIRM's goal to accelerate therapy approvals, the program shows how public-private collaboration can transform discovery into impactful treatments—reinforcing California's leadership in regenerative medicine and delivering progress for patients in need.

A New Life for a Patient with Cystinosis

At 18 months old, Kurt Gillenberg (pictured) was diagnosed with cystinosis, a rare genetic disease causing harmful cystine buildup that damages organs and often leads to early death. By age 32, he had undergone 18 surgeries and multiple kidney transplants. “When I was first diagnosed, doctors told my parents that even though they could treat the disease, it would eventually kill me,” he said.

In 2022, Gillenberg joined a CIRM-funded clinical trial led by Stephanie Cherqui, PhD, testing a one-time gene therapy that modifies blood stem cells to produce the missing cystinosis protein, aiming to reduce cystine buildup and prevent further damage.



Since the trial, Kurt said he has significant improvements. “I’ve never been stronger, and my strength and stamina in the gym increased dramatically,” he said.

This is one of many rare disease clinical trials supported by CIRM, filling a gap often left by major pharmaceutical companies that overlook such conditions due to limited profit potential.

Kurt said he values this potential lifelong cure and the support behind it. “I really appreciate the organizations and individuals who fund research for diseases like cystinosis. Without them, trials like these would not be possible.”



A gathering at UC San Diego’s inaugural Gene Therapy Initiative Symposium in September brought together leading researchers, industry partners, and patient advocates to spotlight breakthroughs in gene therapies for rare and common diseases. The event was supported by a CIRM sponsorship and a gift from Geoffrey and Nancy Stack, whose daughter also participated in the CIRM-funded clinical trial for cystinosis. Stephanie Cherqui, PhD, and Alysson Muotri, PhD, of UC San Diego are leading the Initiative.

Jana K. Dickter, MD, Paul Edmonds, and Monzir M. Al Malki, MD. In July 2022, City of Hope announced Paul Edmonds was in remission from both HIV and leukemia following a stem cell transplant. CIRM's Alpha Clinics Network played a behind-the-scenes role, supporting Institutional Review Board approval, donor coordination, and blood analysis.



Expanding Access and Affordability in Regenerative Medicine

CIRM's mission goes beyond developing cutting-edge stem cell and gene therapies—it also includes making sure these treatments are accessible and affordable for Californians and others who need them.

As more therapies move toward approval, lowering barriers to access and affordability is essential to realizing their full potential. In the past year, CIRM launched several initiatives focused on this goal, including the Patient

Support Program, expanded legislative outreach, and new toolkits and resources to support newly launched programs.

By including access and affordability planning within our clinical research programs, CIRM is aiming to lighten financial burdens, and ensure patients aren't left behind because of cost or limited availability. These efforts are key to making regenerative medicine work for all of California.



Breaking Down Barriers to Clinical Trial Participation

In 2024, CIRM launched the Patient Support Program (PSP), aimed at reducing the logistical and financial barriers that often prevent patients from participating in clinical trials.

Recognizing that stem cell and gene therapy trials must be accessible to truly make a difference, CIRM approved a \$2.5 million award to EVERSANA—a leading provider of global commercial services to the life sciences industry—to establish and implement the PSP. It offers personalized support services to help patients and caregivers navigate the challenges of trial enrollment and participation, ensuring

broader access to CIRM-funded clinical trials.

Eligible patients in CIRM-funded clinical trials may receive assistance with travel, lodging, meals, and other essential needs. The goal is clear: to ease the burden on patients so they can focus on their health and access potentially life-changing treatments regardless of income, background, or location.

The PSP reflects CIRM's broader commitment to equity in research and care. By addressing patients' financial challenges, the program aims to ensure that all Californians who may benefit can participate in clinical trials.

With funding support from CIRM, Children's Hospital Los Angeles doctors are advancing cell and gene therapy pediatric care.



Alpha Clinics Network: A Model for Access

The CIRM Alpha Clinics Network features nine world class medical centers throughout California with a common goal of streamlining and accelerating the delivery of cell and gene therapies to patients who need them.

Children's Hospital Los Angeles (CHLA), a network member in partnership with University of Southern California, now offers ten FDA-approved cell and gene therapies. CHLA is among the most comprehensive pediatric providers on the West Coast, offering treatments for serious conditions such as leukemia, spinal muscular atrophy, hemophilia, sickle cell disorder and inherited vision loss.

This achievement at CHLA underscores the impact CIRM's Alpha Clinics provide in expanding access and delivery of therapies for patients throughout the state.

The Network's 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

Highlights from the 2024 CIRM Alpha Clinics Symposium

CIRM sponsored the 7th Annual Alpha Clinics Network Symposium last year in West Hollywood, drawing over 150 attendees including researchers, clinicians, FDA officials, and patient advocates. Hosted by Cedars-Sinai, the event spotlighted advances in cell and gene therapy, while emphasizing the ways in which CIRM is making these treatments more accessible and affordable for patients.

The event featured in-depth discussions on improving clinical trial access, clinical trial design, and emerging approaches to manufacturing therapies at the point of care.

Keynote speaker Lauren Miller Rogen (pictured)—co-founder of Hilarity for Charity and former CIRM Board Member—spoke about the importance of access to therapies and support services for patients and their families.

“As someone who has been through the heartbreaking journey of losing a loved one to an incurable disease, I can tell you that hope is one of the most powerful things we have. The work being done by CIRM and the Alpha Clinics gives people hope—hope that someday, families like mine won’t have to face the devastating reality of diseases like Alzheimer’s,” she said during her speech.





Engaging Policymakers to Champion Research

In 2024, CIRM boosted its outreach efforts to inform and engage policymakers in both Sacramento and Washington, D.C., ensuring that decision-makers understand the potential of regenerative medicine and the critical role CIRM plays in advancing it.

Through a series of meetings and targeted communications, CIRM leadership—led by Maria Bonneville, Chair of the Access and Affordability Working Group and Vice Chair of the CIRM Board—met with legislators, agency officials, and staffers to highlight the impact of CIRM-funded research and training programs across California.

These efforts demonstrated how public investment in regenerative medicine is accelerating scientific progress, driving economic growth, and offering hope to patients.

At the start of 2025, CIRM Chief Science Officer Rosa Canet-Avilés, PhD, attended the White House Cell and Gene Therapy Forum, which brought together leaders across science, government, industry, and patient advocacy to drive action in accelerating equitable access to cell and gene therapies. Concrete solutions emerged from this meeting, including public-private manufacturing collaborations and streamlined pathways for ultra-rare disease treatment approvals.



Vice Chair of the CIRM Board Maria Bonneville in Washington D.C.



Meeting Communities Where They Are

In 2025, CIRM expanded its community outreach to better inform Californians about the potential of regenerative medicine and how it can be used to treat serious diseases.

Through speaking invitations and partnerships with local organizations, CIRM delivered accessible, multilingual information about regenerative medicine research and clinical trials. These efforts are intended to build trust, make it easier to participate in research, and highlight CIRM's impact across the state.

By working directly with patient groups and community leaders, CIRM ensured its outreach was relevant, inclusive, and responsive to community needs.

CIRM Community Outreach Manager Aditi Desai, MPH, CPH, speaks to attendees at the Parkinson's Moving Day in San Francisco.



CIRM team at the Walk to End Alzheimer's in Fresno



CIRM team at the East Bay Walk & Roll to Cure ALS event in Richmond



▶ Trainees from the CIRM-funded San José State University Stem Cell Internships in Laboratory-based Learning (SCILL) program talk to the public at a campus event.

Empowering California's Future Workforce

California is home to the nation's largest biotechnology workforce, and biotech companies are rapidly advancing the research, development, and manufacturing of cell and gene therapies. To fully realize the promise of these breakthroughs, California must continue building a robust and highly skilled scientific workforce to meet growing industry demands.

Four CIRM-funded education and training programs—SPARK, Bridges, COMPASS, and Scholars—support the next generation of regenerative medicine scientists from high school students to postdoctoral fellows. Each

program offers hands-on experience that builds the technical skills critical to future scientific and medical advancement.

To drive forward CIRM's education network strategy, the agency launched the CIRM Hub (found at cirmhub.cirm.ca.gov) to enable the creation and dissemination of knowledge, scientific tools and resources, and training opportunities to further develop the regenerative medicine workforce in California. This effort is part of multiple on-ramps that leverage and build on CIRM's investments in research, education, and infrastructure.



Josh Nelson spent two decades working in entertainment before deciding to pursue a path in science and medicine. Through the CIRM Bridges Program at Cal Poly Humboldt, he gained hands-on research experience at the Gladstone Institutes, where he expanded his biomedical knowledge in a world-class environment.

Josh credits the program with giving him the opportunity to contribute to cutting-edge regenerative medicine research while preparing for a future in medicine. He is now continuing that journey as a medical student at PNW University in Washington, a testament to the Bridges Program's impact in opening doors for aspiring scientists across California.

CIRM's Education and Training Programs

To date, CIRM's education and training programs have supported more than 4,600 interns, fellows, and trainees. Programs include:

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.

BRIDGES

Bridges to Stem Cell Research and Therapy

15 | PROGRAMS
STATEWIDE

Bridges prepares undergraduate and Master's degrees students from state universities and community colleges for research and career opportunities in regenerative medicine.

COMPASS

Creating Opportunities Through Mentorship and Partnership Across Stem Cell Science

16 | PROGRAMS
STATEWIDE

COMPASS fills a pressing need to mentor and nurture undergraduate students for careers in regenerative medicine. It helps build a skilled workforce in California to help advance research and innovation in healthcare.

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 postdoctoral, and clinical fellows. Scholars contribute to the expansion of regenerative medicine research as they become skilled researchers and innovative leaders.



A New Path in Science

Esmeralda “Esme” Ruiz-Garcia grew up in a Mexican-American family where working in healthcare was a shared tradition. Though she always loved science, she didn’t see herself following the traditional medical path. As the first in her family to attend college, she initially pursued nursing but soon realized it wasn’t the right fit.

Her journey led her to California State University, San Bernardino, where Nicole Bournias-Vardiabasis, PhD, introduced her to biomedical research. That spark led Esme to join the CIRM-funded Bridges to Stem Cell Research program, where she worked on Alzheimer’s research and then interned in the UC Riverside bioengineering lab of Huinan Liu, PhD. Despite the steep learning curve, Esme found her calling.

“I fell in love with engineering, I didn’t really understand what I was getting myself into but I’m forever and eternally grateful that I gave myself the opportunity to get out of my comfort zone.”

— **Esmeralda Ruiz-Garcia,**
UC Riverside Student

Today, Esme is a second-year PhD student in the chemical and environmental engineering lab of Ke Du, PhD, at UC Riverside. She credits the CIRM Bridges program for helping her discover a world of possibilities in science and engineering.

“It isn’t a one-way road,” Esme said. “There are thousands of roads I can take that will all lead me toward helping others.”

Cultivating Tomorrow's Scientific Leaders

Last year, CIRM hosted two events that brought together emerging scientists from CIRM's education and training programs to network, learn, and inspire one another.

First, the inaugural Trainee Networking Conference at the University of Southern California welcomed over 400 trainees from CIRM's Bridges, COMPASS, and Scholars programs. Featuring 85 speakers, 44 panelists, 76 trainee presentations, and more than 250 trainee posters, the event offered an environment for trainees of different education levels to participate in scientific exchange, networking, and professional development opportunities.

In addition to hearing from leading researchers about their cutting-edge science, trainees also took part in interactive career panels and small group workshops covering topics such as scientific publishing, biotech paths, and applying for graduate schools. Trainees further heard from patient advocates who

shared personal stories, underscoring the real-world impact of their research in regenerative medicine.

Earlier in the summer, CIRM's annual SPARK Conference brought more than 100 high school interns together at UC Riverside. These SPARK interns presented their summer projects and connected with peers and mentors in a supportive and highly interactive atmosphere.

An intern from the UC Davis SPARK cohort said, "SPARK has been a truly unforgettable experience and has allowed us to fully embrace the beautiful world in all its unknown glory as we delved into the realm of scientific research. It's intricate, it's perplexing, it's hard, it's wonderful, it's awe-inspiring, it's powerful—a perfect mixture of everything we've ever wanted to seek."

Together, these conferences underscore how CIRM is nurturing California's next generation of scientific leaders and empowering them to advance CIRM's vision.

2024 SPARK high school interns and program directors

CIRM President & CEO Jonathan Thomas, PhD, JD, speaks to trainees from UC Riverside at the 2024 SPARK conference.

Over 250 research posters were showcased during the inaugural CIRM Trainee Networking Conference, held last year at the University of Southern California.

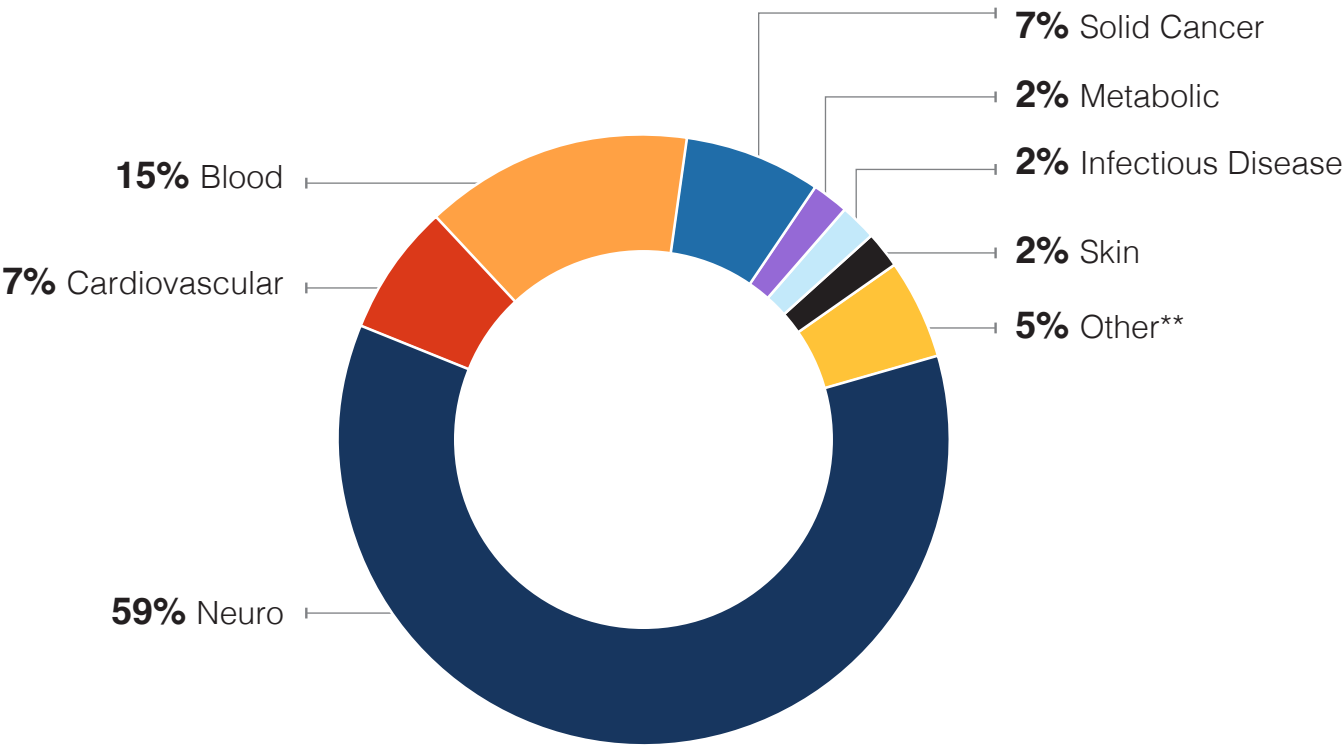


Research Portfolio Snapshot

During the 2024–2025 fiscal year, CIRM continued to advance its mission by funding a diverse array of research projects across multiple disease areas. This portfolio reflects our commitment to supporting discovery, translational, and clinical-stage research that addresses both prevalent and rare diseases and conditions.

CIRM-Funded Research Portfolio by Disease Area

Total research awards for fiscal year 2024-2025: 41*



Percentages are normalized to the total number of Research awards approved during the 2024-2025 fiscal year. Research includes discovery, translational, and clinical research awards.

Percentages rounded to the nearest whole number.

*The 41 awards include DISC2, DISC4 (neuro focus), TRAN, CLIN1, and CLIN2 programs. New calls for applications were paused to ensure alignment with updated Strategic Allocation Framework priorities and program structures. DISC0 was postponed to fiscal year 2025-2026 due to the January 2025 Southern California wildfires.

**Other includes: transplant tolerance and technology/tools.

Budget Update

CIRM’s activities 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 2024-25: \$489.9 million

Research Budget for Fiscal Year 2024-25:*	\$	458.3 million
Administrative Budget for Fiscal Year 2024-2025:	\$	31.6 million
Funds Approved (awards):	\$	308.3 million
Funds Remaining:	\$	150 million
Balance Under Active Management:	\$	624 million
Number of Active Project Awards:		324

Approved Budget for Fiscal Year 2025-26: \$505.7 million



Discovery Research

\$158.2 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

\$160 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

\$135 million

CIRM is building a world-class therapeutics portfolio and supporting each project with resources to increase its chances of success into and through clinical trials.



Education Programs

\$1.5 million

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

\$51 million

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

*ICOC approved two 6-month budgets in FY 24-25 which included a carry forward between periods.

**Now known as Preclinical Development



Leading with Vision & Impact

CIRM's progress is driven by the leadership of its Board (also known as the Independent Citizens' Oversight Committee), whose experiences doing research, treating patients, and being patients themselves inspire them to put the people of California at the center of every decision.

The Board's actions on new funding programs, research and clinical support, and research policies, are guided by the recently adopted Strategic Allocation Framework that provides a roadmap for how the agency will allocate remaining funds to drive research discoveries to patients. This Framework ensures CIRM funds bring maximum benefit to people in California who are waiting for cures.

In the past year, CIRM welcomed six new Board members whose diverse expertise strengthens our leadership: John Carethers, MD; Jeffrey Golden, MD; Hala Madanat, PhD, MS; Donald Taylor, PhD, MBA, CLP; Shauna Stark, MS; and Yael Wyte, MSW. At the same time, CIRM staff continued to implement programs, engage communities, and manage grants with transparency and impact.

"We all have hope, and we all want to find the cure for a disease or all diseases. That's part of our human core," said Wyte.

With patients at the heart of every decision, CIRM's Board remains committed to innovation, collaboration, and improving lives across California.