



CIRM 2020-2021 Update to the CFAOC

Completion of Prop 71 Programs and Launch of Prop 14

Maria T. Millan, M.D.
CIRM President and CEO
November 10, 2021

CIRM: Created by Prop 71 and continuing under Prop 14

Proposition 71
2004

\$3B Bond Funding

CIRM

Accelerating, Patient-Centric,
Funder, Partner and De-risker for
Basic, Translational, and Clinical Research
Infrastructure and Education Programs

Proposition 14
2020

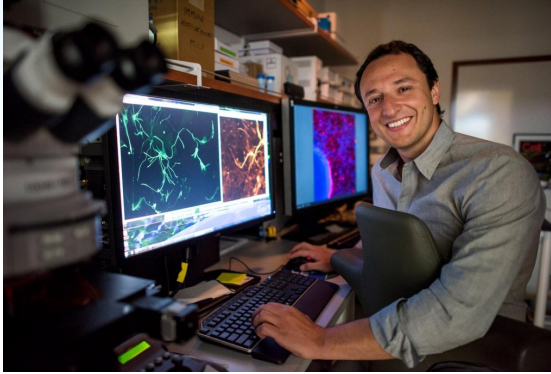
\$5.5B Bond Funding

CIRM | Our Identity and Impact



- Funded **>1000 projects**
- Advanced stem cell research and therapy development spanning a broad range of diseases
- Funded **70+ clinical trials with 2700+ enrolled**
 - from first-in-human to pivotal phase 3
 - from common diseases and rare disease
 - demonstrated that cures are possible (40 children treated with cell-gene therapy for ADA-SCID)

CIRM | Our Identity and Impact



- Generated **3,000 peer-reviewed publications** describing scientific and medical discoveries
- Funded **12 stem cell research facilities** and **17 shared research laboratories**
- Built the **largest iPSC research bank** with >2600 cell lines for modeling
- Enabled **invention of research and translational tools**
- Created **novel genomic datasets and bioinformatics tools for stem cell research**

CIRM | Our Identity and Impact



- Supported research that led to **90+ candidates** advancing to clinical development.
- Established **5 Alpha Stem Cell Clinics** that have accelerated execution of 100+ stem cell clinical trials.
- Attained **Expedited Pathway** from FDA 15% of RMATs.
- Supported **developmental progression of 67 therapies** via successive CIRM awards.
- Accelerated **73% of funded IND-enabling programs** to initiate clinical trials within 2 years.

CIRM | Our Identity and Impact



- Trained over **3,000 students and scholars** to become the future workforce of regenerative medicine.
- Stimulated the CA economy with **\$10.7B of gross output and 56,000 new FTE jobs** created during the 2004-2018 period.
- Helped enable CIRM-funded projects to attract **\$18B+ of industry funding**.

CIRM Investments

2020-2021 Investments



INFRASTRUCTURE

\$0 M



EDUCATION

\$208K



DISCOVERY

\$4.5M



TRANSLATION

\$14.5M



CLINICAL

\$56.4M

Total Investments since 2004

\$480M



\$356M



\$922M



\$371M

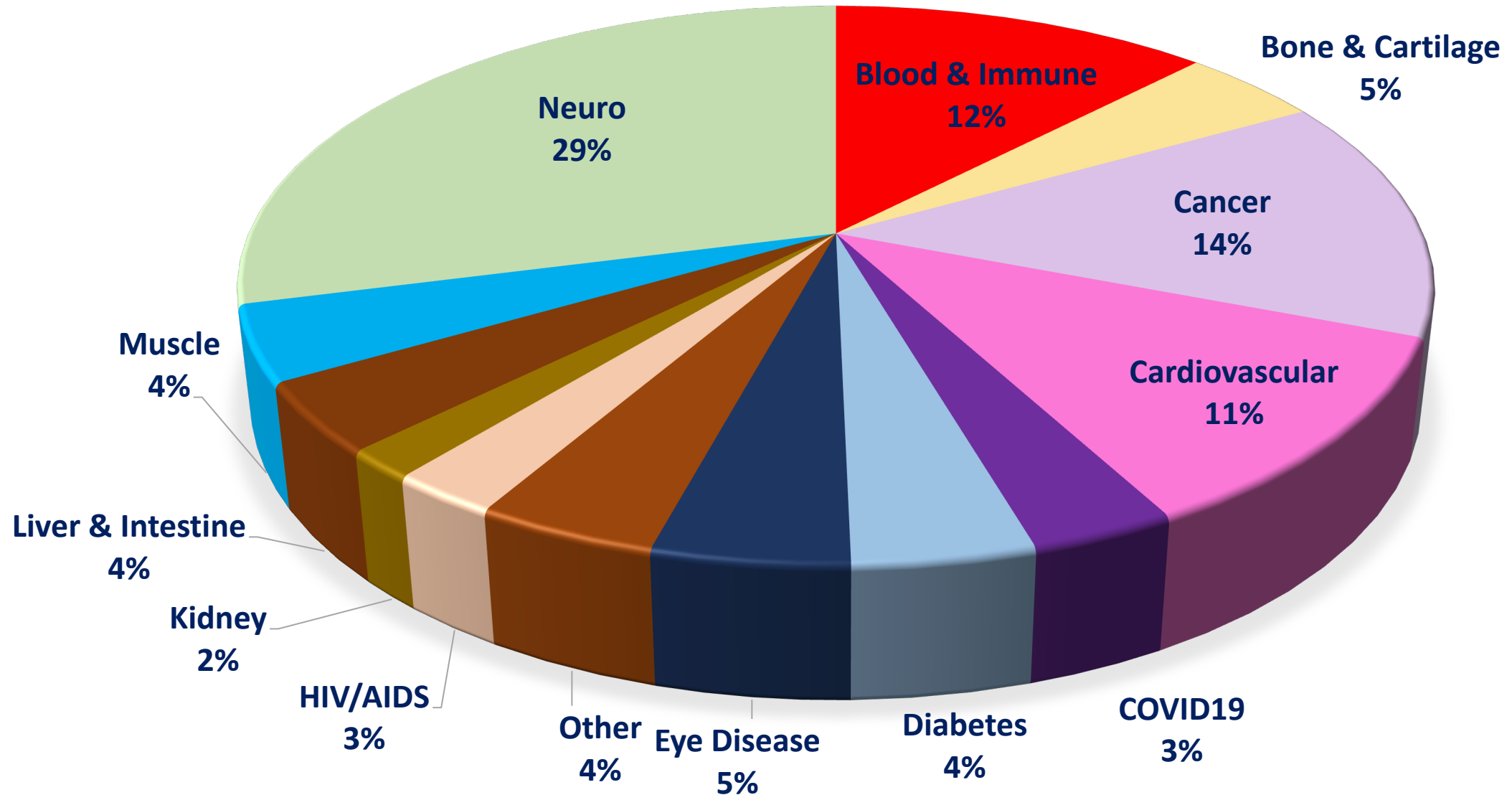


\$840M



CIRM Research & Development Portfolio

596 Grants, excluding non-disease focused awards



CIRM Clinical Portfolio: **Meningocele (spinabifida)**

A stem cell seeded patch to encourage tissue regeneration and protect the spine from damage

- Placental mesenchymal stem cell-seeded dural matrix.
- 6 patients treated with seeded patches during *in utero* surgery to repair the spinal defect.
- CIRM is funding the Phase 1 portion of a Phase 1/2 trial. Newborns are monitored postnatally, assessing safety and efficacy outcomes.



PI: Diana Farmer, UCD
CIRM Funding \$ 8,996,474

CIRM Clinical Portfolio: **Pediatric Brain Cancer**

CAR-T immunotherapy to treat pediatric patients with refractory or recurrent brain cancer

- CAR-T targeting brain tumor antigen IL13Ra2
- 19 pediatric patients treated with infusions into CSF
- Lymphodepletion is introduced in the second part of the study.



CIRM
CALIFORNIA STEM CELL AGENCY

PI: Leo Wang, City of Hope

CIRM Funding \$8,401,309

CIRM Discovery Portfolio: **Muscle Waste/Atrophy**

Stimulating endogenous muscle stem cells to counter muscle wasting

Discovery award– novel therapeutic approach to induce muscle regeneration

Goal– Selection of a candidate muscle stem cell targeting therapeutic for the treatment of COVID-19 associated diaphragm atrophy.



PI: Helen Blau, Stanford

CIRM Funding \$2,615, 743

CIRM Discovery Portfolio: **Leukoencephalopathy**

Transplantation of Pluripotent Stem Cell Derived Microglia for the Treatment of Adult-onset Leukoencephalopathy (HDLS/ALSP)

Discovery Award: Leveraging protocol developed through a previous Tools and Technology award

Technology: human pluripotent stem cell-derived microglial cell transplantation candidate to treat a rare genetic neurodegenerative disease (Adult onset leukodystrophy)



PI: Mathew Blurton-Jones, UCI
CIRM Funding \$1,362,264

CIRM Discovery Portfolio: Predicting Cardiotoxicity

Safety “Clinical Trial” of the Cardiac Liability of COVID19 Polytherapy

Discovery award – Optimization and validation of a human “heart on a chip”

Technology: microphysiological system (MPS) for more accurate drug screening and prediction of pro-arrhythmic risk.

Purpose: Predict toxicity to the heart especially in patients with diverse backgrounds infected and treated by multiple agents for COVID-19



PI: Kevin Healy, UC Berkeley/Organos

CIRM Funding \$1,124,081

CIRM Activities 2020-2021

- The first half of the 2020-2021 fiscal year was a “wind down” of the final 5-year Strategic Plan under Prop 71
- CIRM successfully exceeded the final 5-year Strategic Goals under Prop 71 in December 2020
- With the Passage of Prop 14 in November 2020, CIRM has been in a period of growth and transition during the remainder of the 2020-2021 budget
- 2021 Activities:
 - ✓ Relaunched funding in education, discovery, translational, clinical trials and supplement for Alpha Clinics
 - ✓ Strategic Plan development for CIRM under Prop 14- Draft Presented in Oct. 2021 with Final Plan for Board approval in Dec. 2021
 - ✓ Build a new team to support an increased operations needs and upgrades and the strategic plan development and launch



Internal CIRM project teams

Increase

Patient Access to
Innovative Treatments

Advance

World-Class Science

**Strategic
Themes**

Maximize

Impact through
Operational Excellence

Build

Pathways to
Commercialization

CIRM | Path to Strategic Plan



Broad Stakeholder Input

- 2020-2021 ICOC Meetings
- Stakeholder Town Hall Meeting and Survey
- Strategic Scientific Advisory Panel Meeting
- GWG Program Meeting
- CIRM Program Meetings
 - Grantee meeting
 - Alpha Clinics Symposium
 - Bridges & SPARK meetings

Patient & Community

- Patient Navigation Roundtable
- Alpha Clinics Symposium: Patient Access & DEI Panel

Knowledge Networks

- Grantee Meeting: Knowledge Networks Panel
- Data Biosphere Planning Committee Meeting
- Shared Labs Survey

Manufacturing & Commercialization

- Industry Alliance Program Meeting
- Industry Partner Day
- Manufacturing in California Workshop
- GMP Manufacturing Facility Survey

CIRM | Evolving our Mission Statement



From

Accelerating stem cell treatments to patients with unmet medical needs.

To

Accelerating world-class science to deliver transformative regenerative medicine treatments to California and worldwide.

CIRM | New Mission Statement



***World Class Science:** Accelerating through our funding program, partnership model and “Team Science”*

[Accelerating world-class science]
[to deliver transformative regenerative medicine treatments]
[to California and worldwide.]

***Transformative:** creation of novel health care delivery models and a trained workforce that bring **real world solutions and cures** to our diverse community*

***Opportunity for All:** Access for diverse communities of California to clinical trials and ensuing treatments, educational and scientific programs*

Strategic Plan Goal: To address the challenges and opportunities of the rapidly advancing regenerative medicine field fostered by CIRM



Advance
World-class Science



Deliver
Real World Solutions



Provide
Opportunity for All

The new strategic plan is designed to **enhance**, **organize** and **interconnect** CIRM's proven funding model to achieve the overarching goals



Education



Infrastructure



Discovery



Translation



Clinical



Advance
World-class Science

Deliver
Real World Solutions

Provide
Opportunity for All

The Regenerative Medicine field is advancing and maturing rapidly

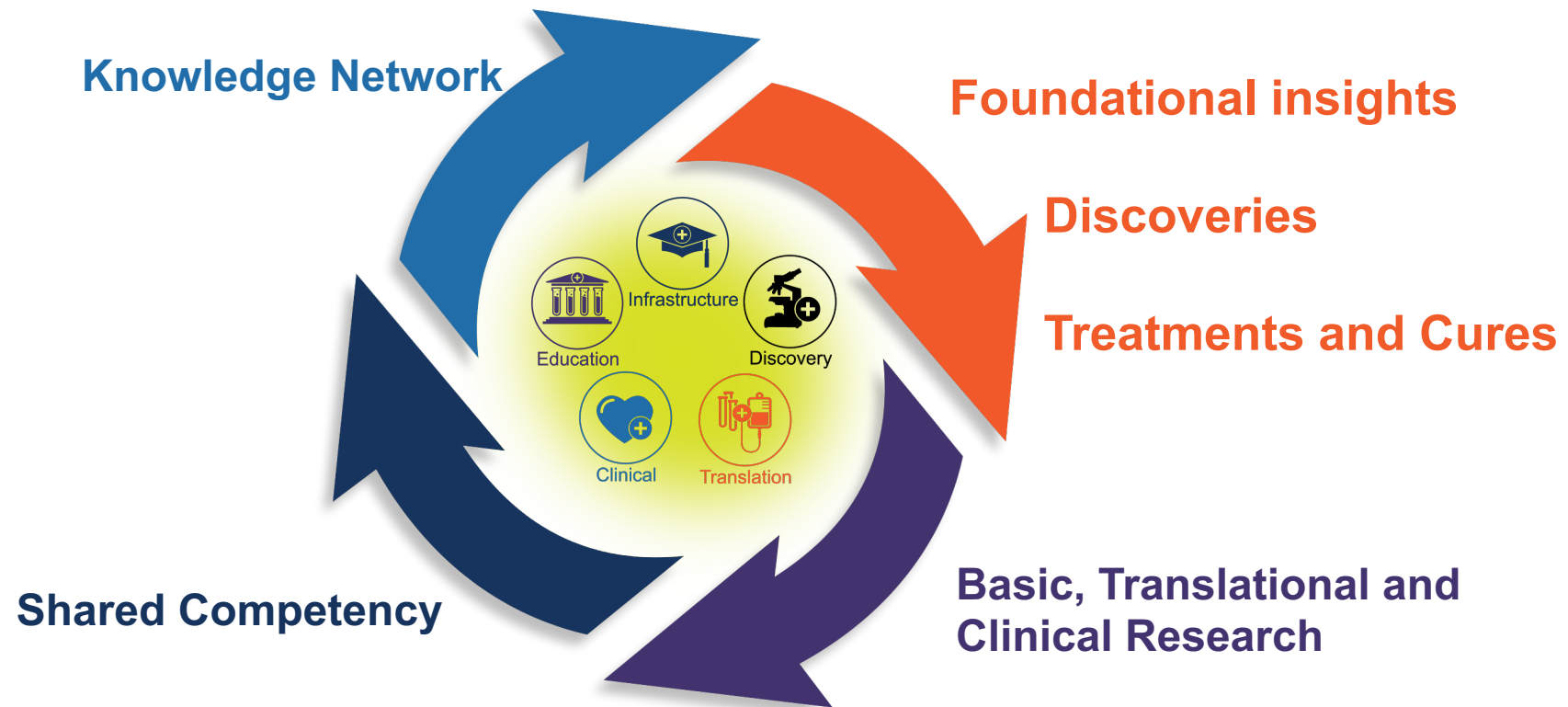
Despite the number of funded programs and data generated there are still limited cures for devastating conditions (including CNS and diseases of the brain*)

Stakeholder Input: CIRM should become a guiding leader in the paradigm shift for biomedical research

- Incentivize and lead the development of collaborative efforts
- Integrate data sharing into CIRM operating principles and funding
- Democratize advanced, specialized resources & standards – (Competency hubs)
- Create knowledge networks
- Leverage a diverse, inclusive and equitable foundation for scientific communities and in science

** Prop 14 earmarks funding for research in CNS and Disease of the Brain*

Create a systemic approach to foster a culture of collaboration, efficient knowledge transfer and diversity and inclusion to strengthen and empower scientific output



The Regenerative Medicine field is advancing and maturing rapidly but pervasive bottlenecks slow clinical development and stall the regulatory approval of potentially transformative therapies

Stakeholder Input:

- Enhance expertise and capacity of clinical research and manufacturing infrastructure
- Accelerate and optimize regulatory, clinical and manufacturing pathways to approval for transformative therapies

CIRM

Deliver Real World Solutions Public-Private Manufacturing Network



CIRM-Funded Academic GMP Facility Network



Industry Partners

- Manufacturing services
- Resources
- Investment and Partnerships



CIRM
CALIFORNIA'S STEM CELL AGENCY

- **Accelerate**
and de-risk path to commercialization
- **Advance**
standards and quality by design
- **Build**
manufacturing leadership and workforce

Expand Alpha Clinics and create Community Care Centers of Excellence to support inclusive clinical research and treatment delivery



*stipulated in Prop 14



The Regenerative Medicine field is advancing and maturing rapidly

Focused and deliberate actions are needed to ensure diverse workforce and patient participation in the discovery, development and delivery of therapies

Stakeholder Input:

- Support education and training programs that will build a diverse highly skilled regenerative medicine workforce
- Develop a strategy to address access and affordability for all patients

Build inclusive participation opportunities for all stakeholders, from the students to the workforce to the patients.

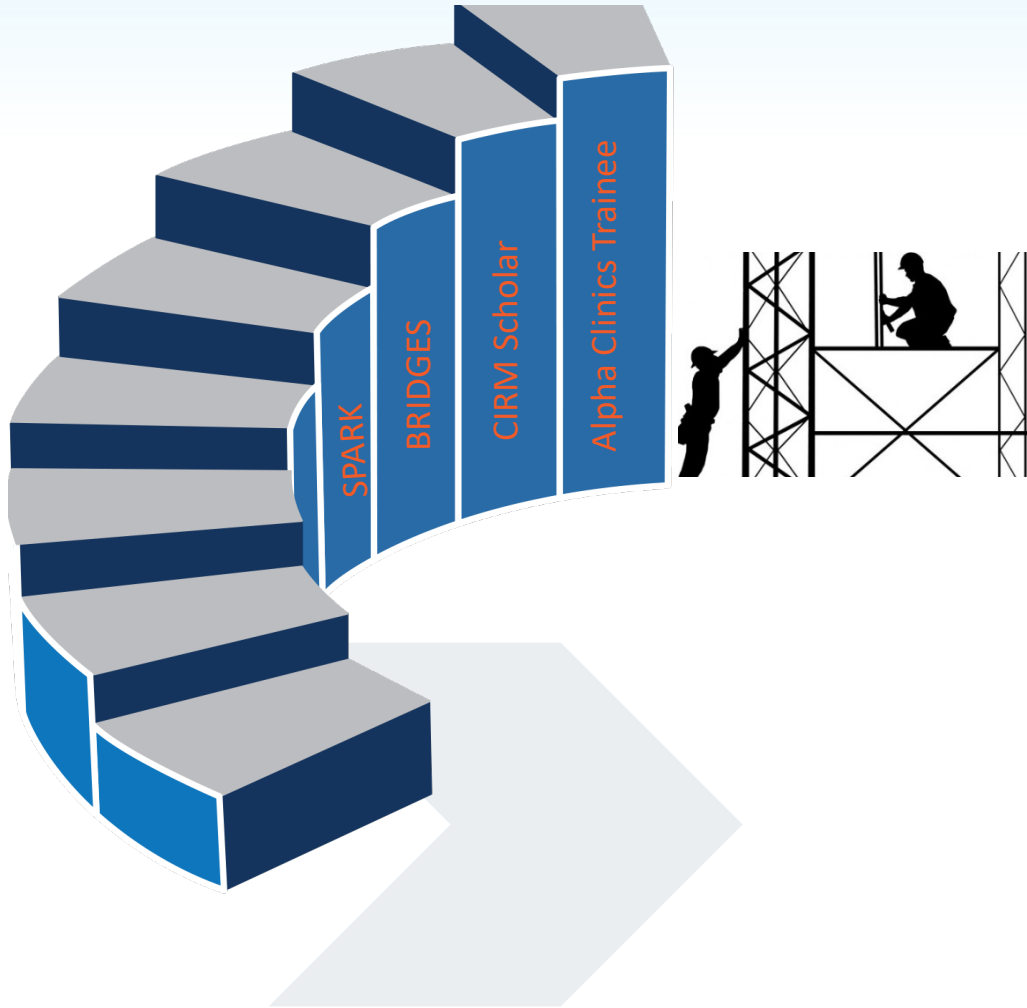


Sickle Cell Trial participant

- **Build a diverse and highly skilled workforce** to support the growing regenerative medicine economy in California*
- **Deliver a roadmap for access and affordability** of regenerative medicine for all California patients*

*stipulated in Prop 14

CIRM | Provide Opportunity for All



- CIRM (re)launched education and training programs in 2021
- Additional concepts under development
- Created multiple on-ramps to develop the next generation of leaders, scientists, clinicians and future workforce
- Training opportunities across CIRM funded programs
 - Basic, translational and clinical research
 - Manufacturing sciences
 - Science Communication
 - Community Engagement

5-Year Strategic Goals

Advance World-class Science

- Develop next-generation **technology competency hubs** that broadly empower and connect California's research ecosystem
- Build **knowledge networks** that foster and advance novel discovery, translational and clinical research approaches

Deliver Real World Solutions

- Optimize CIRM's **clinical trial funding partnership** model to advance more therapies to FDA marketing approval
- Overcome manufacturing hurdles for the delivery of regenerative medicine therapies by building a **public-private manufacturing partnership network**
- Expand **Alpha Clinics and create Community Care Centers of Excellence** that support diverse patient participation in the rapidly maturing regenerative medicine landscape

Provide Opportunity for All

- Build a **diverse and highly skilled workforce** to support the growing regenerative medicine economy in California
- Deliver a **roadmap for access and affordability** of regenerative medicine for all California patients



World-Class Science | Real World Solutions
Opportunity for All