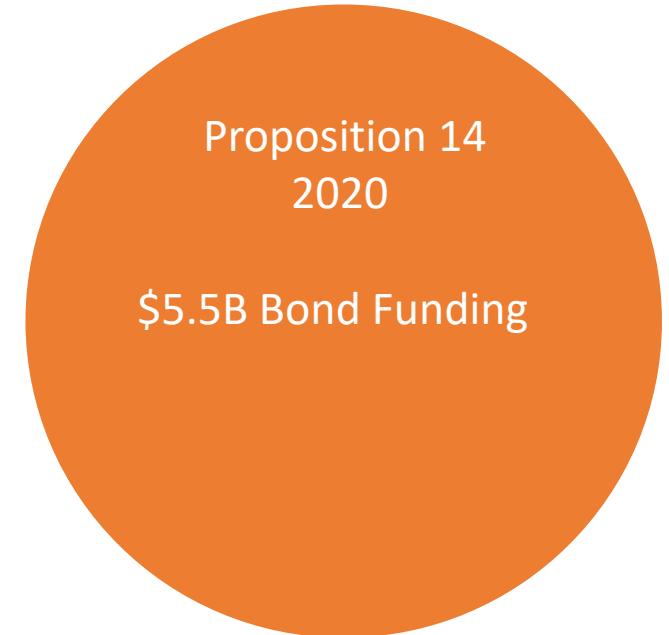




## Draft Strategic Plan for CIRM - Prop 14 Era

Maria T. Millan, M.D.  
President's Report  
ICOC Meeting  
October 19, 2021

# CIRM: Created by Prop 71 and continuing under Prop 14

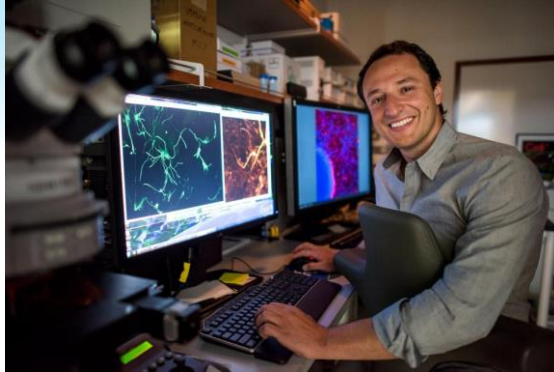


# CIRM | Our Identity and Impact



- 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 to rare diseases
  - 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 total 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 | Path to the Strategic Plan for CIRM- Prop 14 Era

## Internal CIRM project teams



# 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 work force 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 rapidly, generating massive amounts of scientific data but effective treatments are still limited for devastating conditions (including CNS and diseases of the brain\*)**

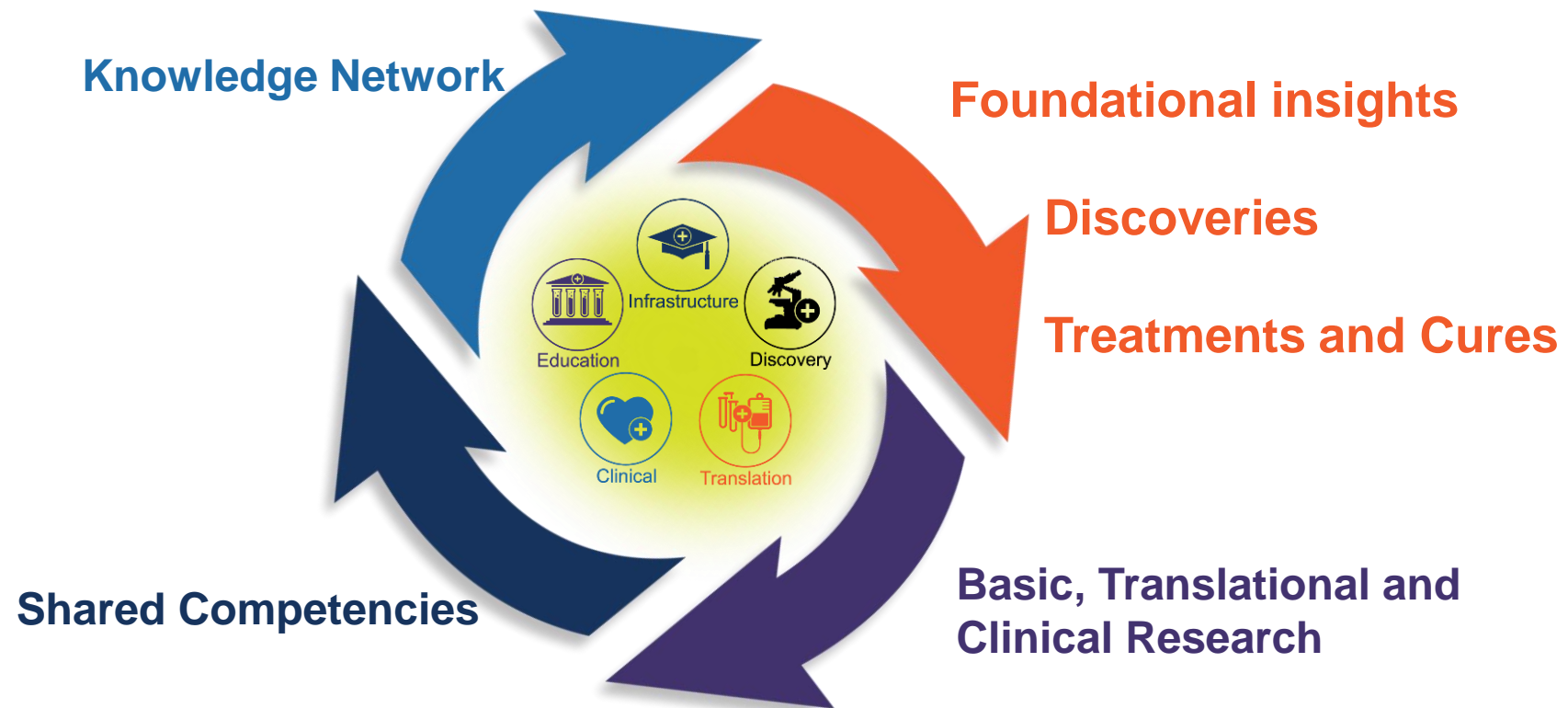
**Stakeholder Input:** CIRM should be a guiding leader in the paradigm shift for biomedical research

- Incentivize and lead the development of collaborative efforts
- Integrate data sharing into CIRM operating principles and funding
- Make available novel technology resources & standards to all – (Competency hubs)
- Create knowledge networks
- Build a solid foundation to promote diversity, equity and inclusion in science

*\* Prop 14 stipulates 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*



*Leverage collective scientific knowledge to inspire collaborative research that addresses Californians' unmet medical needs*



## 5-year Strategic Goals

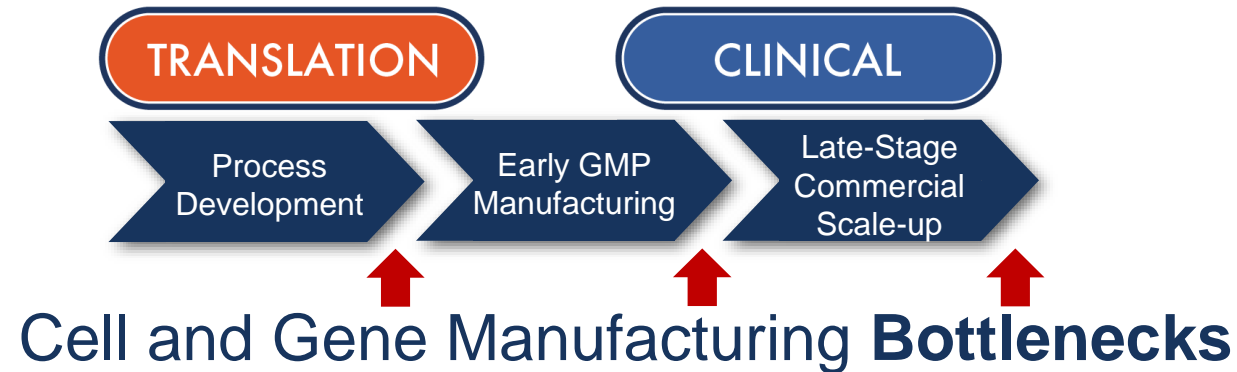
- Develop next-generation **technology competency hubs\*** that broadly empower and connect California's research ecosystem
- Build **knowledge networks** that foster and advance novel discovery, translational and clinical research approaches

\* Shared labs, stipulated in Prop 14, would serve as one form of competency hubs

**The Regenerative Medicine field is advancing 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



## Academic and Industry Input:

- Build a California manufacturing network that accelerates manufacturing development from early translation through to commercialization
- Leverage CIRM's education programs to help academia and industry build faster on-ramps for manufacturing career pathways

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



CIRM

# Deliver Real World Solutions Clinical Research Infrastructure



CIRM's Alpha Clinics Network is a clinical research competency hub that accelerates clinical trial execution.

**100+** academic and industry sponsored clinical trials supported and **750+** patients treated.

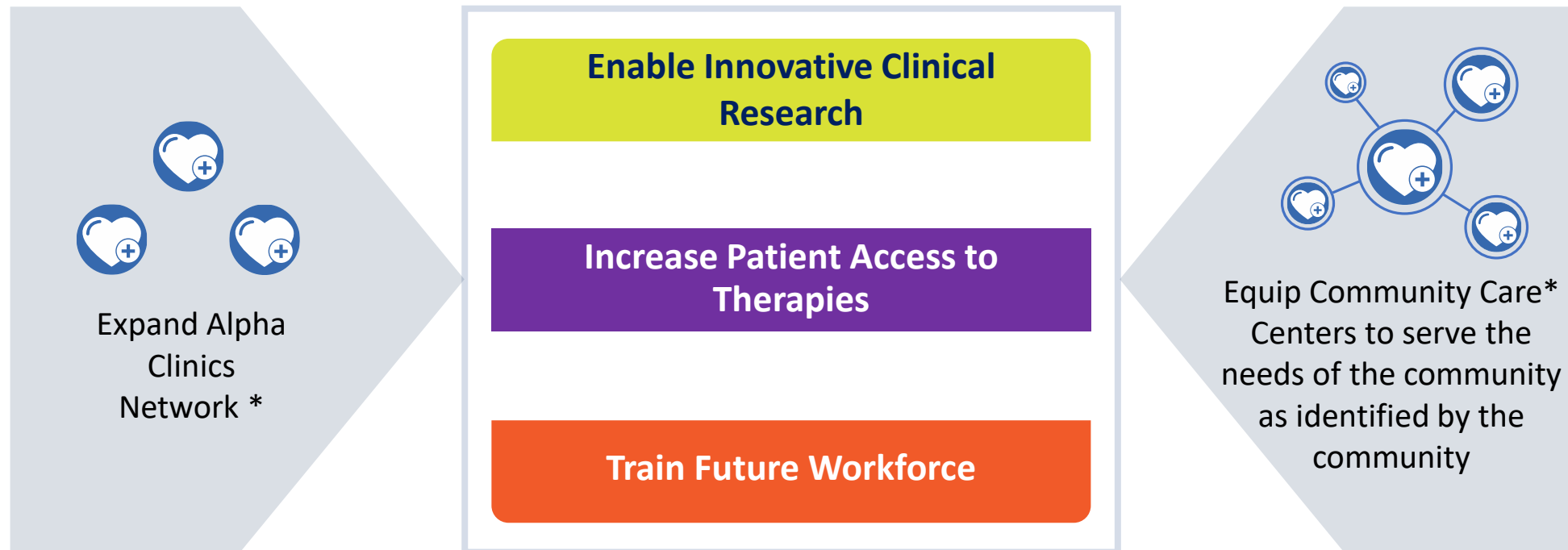
Prop 14 anticipates continued funding and expansion

## Stakeholder Input:

- Need for expanded clinical research expertise and capacity
- Address needs of patients from all California communities
- Meet California's workforce demands by providing specialized training for regenerative medicine delivery



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



\*stipulated in Prop 14

*Overcome critical bottlenecks to accelerate approval of therapies for all patients*



## 5-year Strategic Goals

- **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.

\*Alpha Clinics expansion and Community Care Centers stipulated in Prop 14



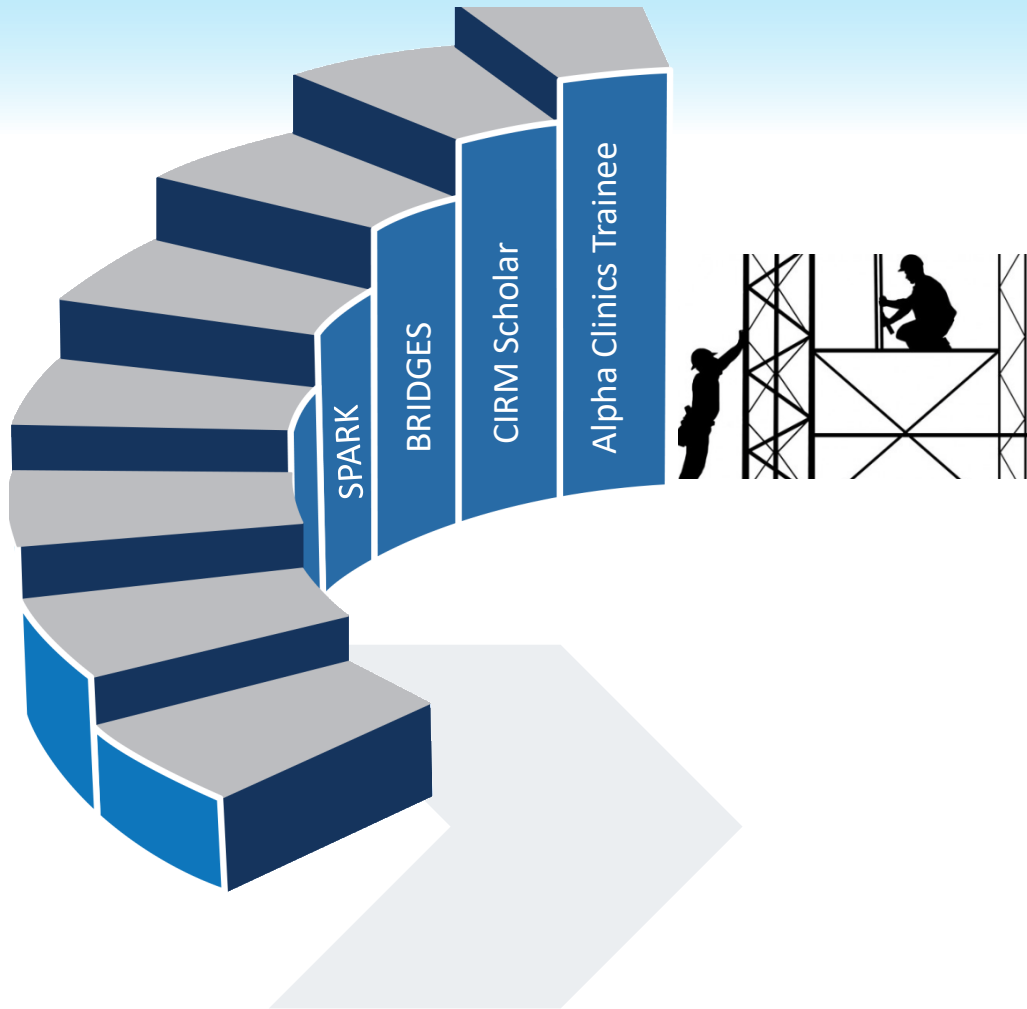
## **The Regenerative Medicine field is advancing and maturing rapidly**

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

# 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



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



## 5-year Strategic Goals

- **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\*

\*Education programs and AAWG are stipulated in Prop 14

# 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**