

# Progress and Prospects: CIRM's Strategic Plan, Evolution, and Forward Outlook

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CEO & President  
CFAOC – May 2024



## OUR MISSION

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







## Funding Allocations

- **Total Bond Provision:** \$5.5 billion to support all CIRM initiatives
- **Targeted Research Funds:** \$1.5 billion specifically for Neurological diseases



## Enhanced Accessibility and Affordability

- **Accessibility and Affordability Working Group (AAWG)** established to facilitate access to CIRM-funded products for all Californians, focus on underserved communities
- **Alpha Clinics and Community Care Centers of Excellence (CCCE):** Expansion to broaden clinical trial access and community care



## Workforce and Infrastructure Development

- **Training Programs:** Initiatives for a skilled workforce in regenerative medicine
- **Shared Labs:** Investments to enhance innovation and service underserved areas



## Governance Enhancements and Financial Oversight

- **Advisory Task Forces:** For strategic direction and policy guidance
- **Revenue Management:** General Fund contributions to offset expenses
- **Operational Transparency:** Mandatory updates on COI and SWG
- **Financial Accountability:** Regular audits for fiscal responsibility



## Operational Parameters

- **Staffing limits:** 70 employees, plus 15 for AAWG



## Advance World Class Science

- Develop shared resources
- Build knowledge networks



## Deliver Real World Solutions

- Advance therapies to marketing approval
- Create a manufacturing partnership network
- Expand Alpha Clinics Network
- Create Community Care Centers of Excellence



## Provide Opportunity for All

- Build a diverse and highly skilled workforce
- Deliver a roadmap for access and affordability

# CIRM's Pillar Programs & Investments

\$4.1B in total grants to date



DISCOVERY



TRANSLATION



CLINICAL



EDUCATION



INFRASTRUCTURE

**Prop 71 + 14 era**

**\$1.1B**

**\$615M**

**\$1.3B**

**\$438M**

**\$578M**

**Prop 14 era**

**\$157M**

**\$189M**

**\$402M**

**\$197M**

**\$89M**

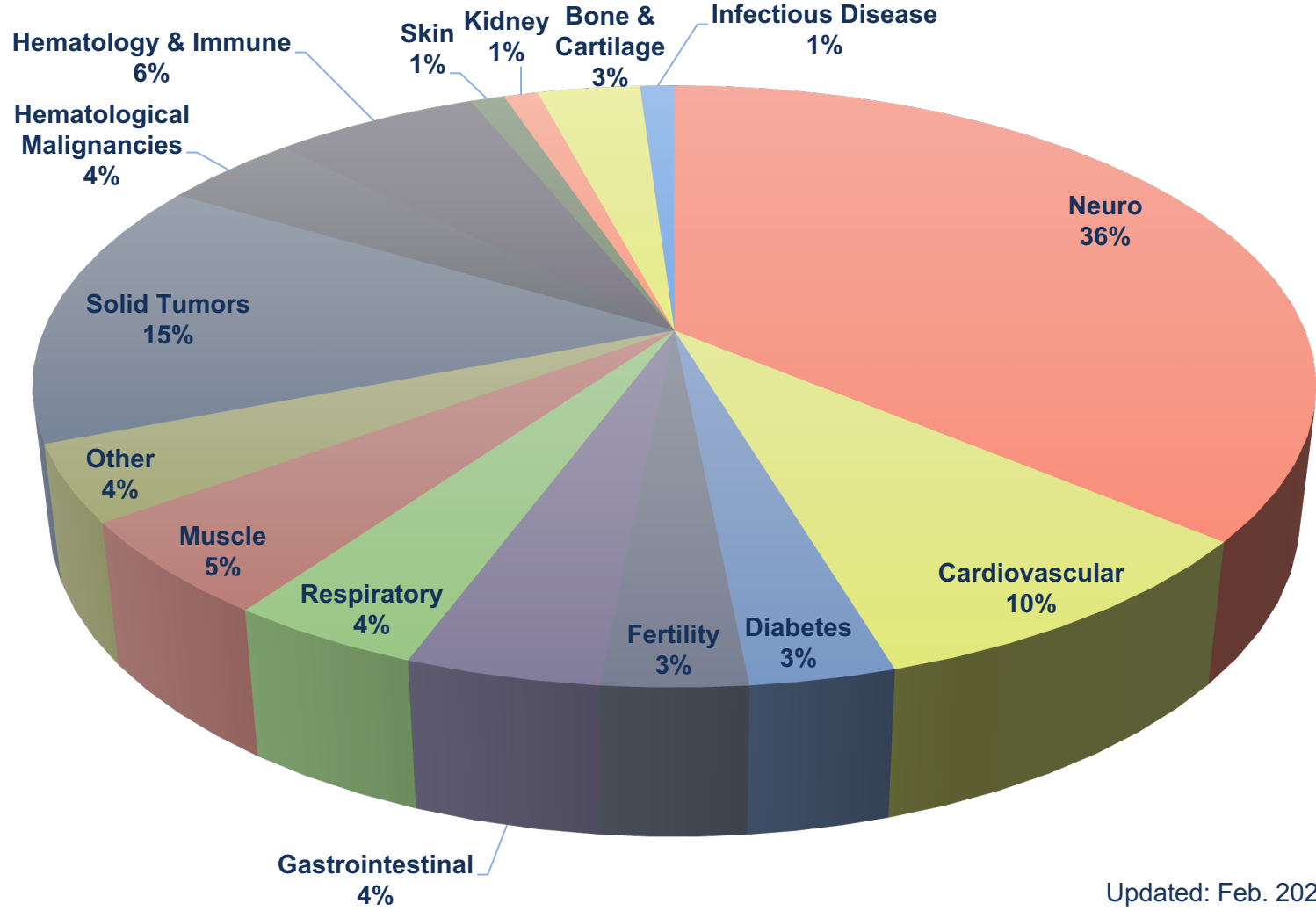
**\$249M** in the neuro space in Prop 14 era



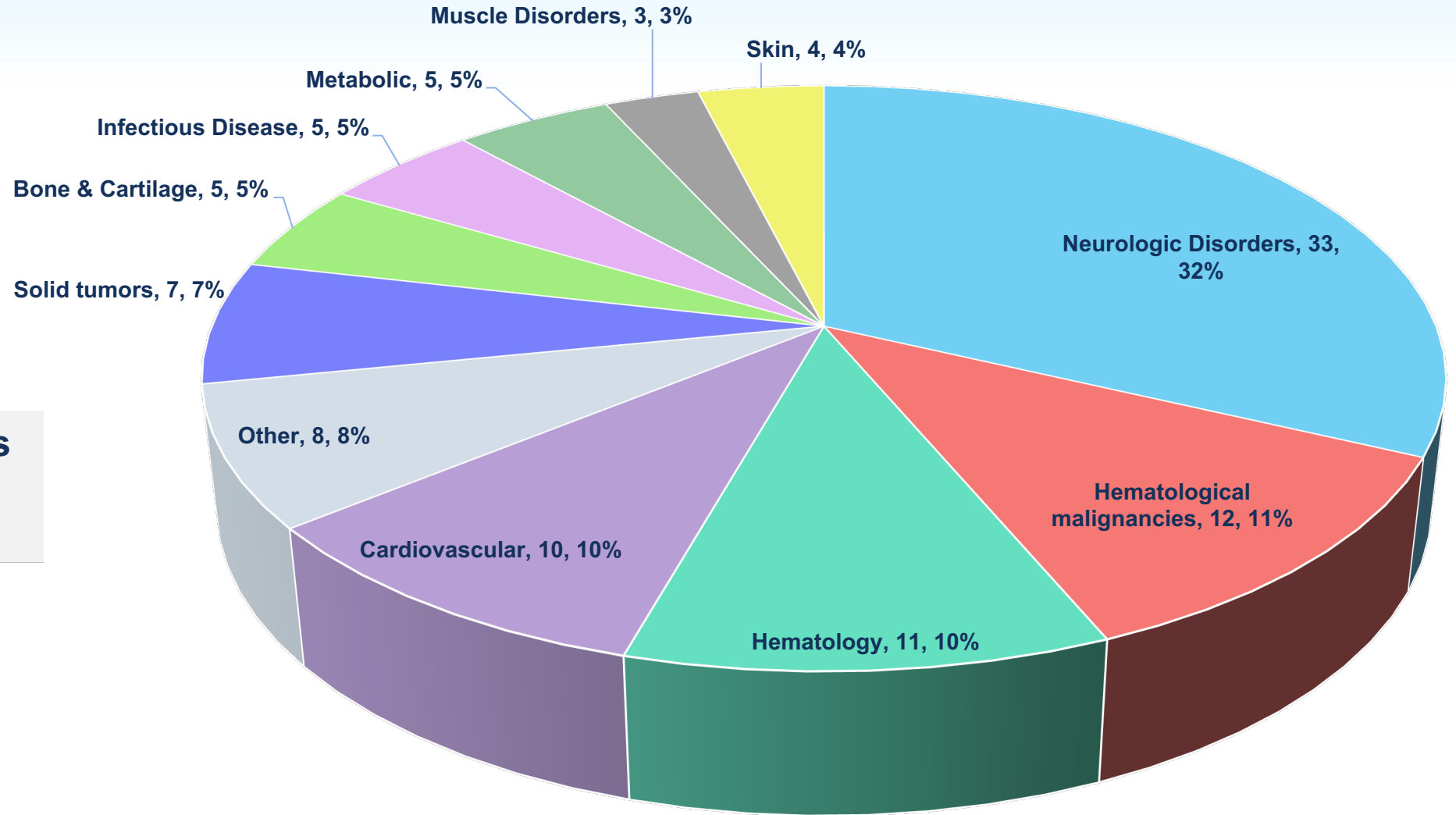
# Current Discovery Portfolio

Foundational	Candidate
27.8%	72.2%

Active DISC Awards (n=97)  
Total DISC awards (n=692)



# Current Translational Portfolio



**Total R&D TRAN awards**  
as of January 31, 2024  
(n=103)

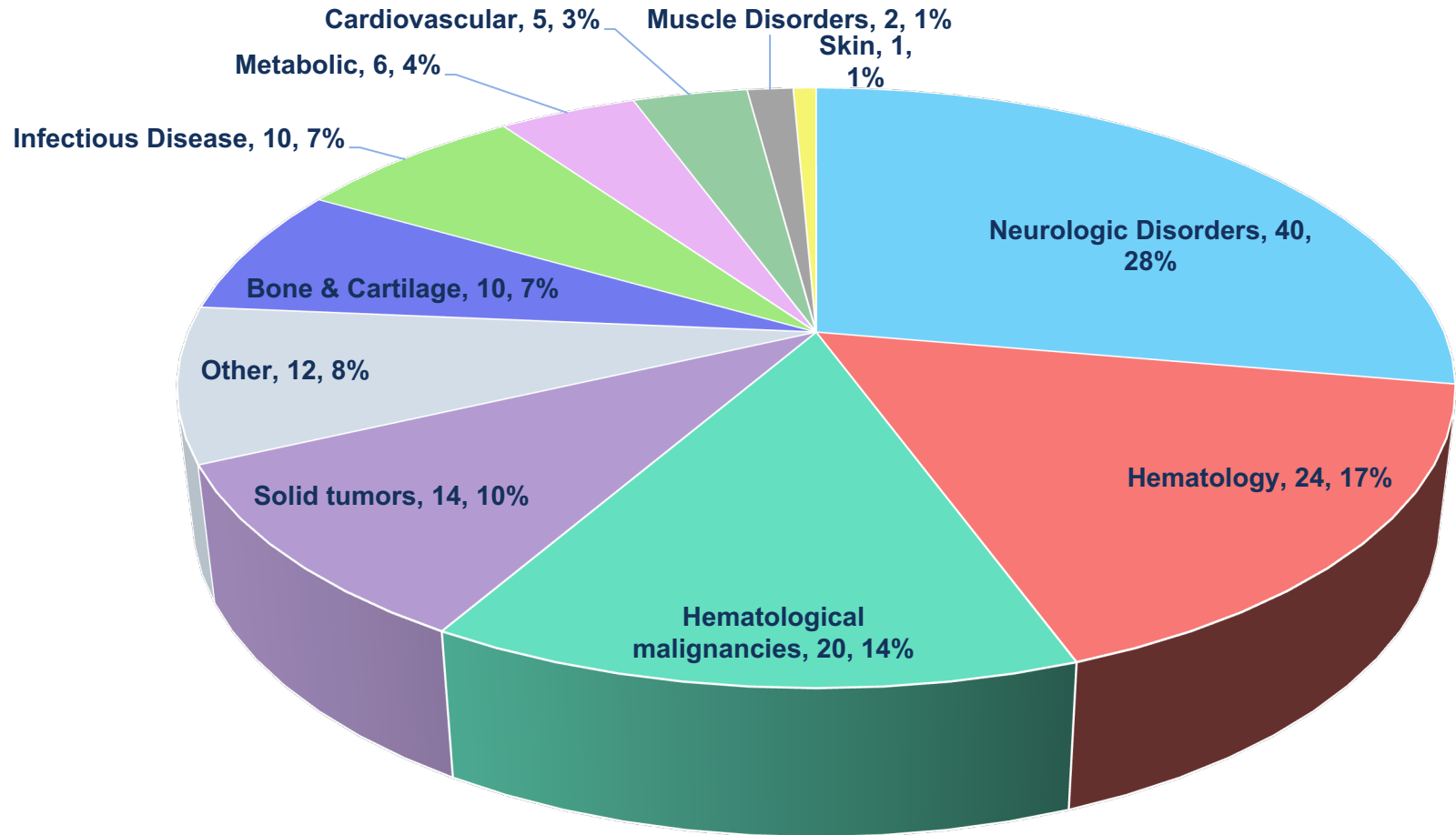


# Current Clinical Portfolio Phases

IND-Enabling	Early Clinical	Mid-Clinical	Pivotal-Late Clinical
30.55%	55.55%	9.02%	4.86%

86% of current portfolio = de-risking projects

**Total R&D CLIN awards**  
as of January 31, 2024  
(n=144)



## CIRM's Impact:



### **Collaborative Networks for Discovery Research**

CIRM is fostering multidisciplinary teams, enhancing understanding of disease mechanisms and leading to discoveries that will push boundaries in treatment development



### **Training and Workforce Development**

CIRM's educational initiatives integrate an educational framework with practical applications, creating a comprehensive network that connects training with real-world application



### **Commercialization of Cell and Gene Therapies**

CIRM is committed to advancing cell and gene therapies from research to FDA marketing approval, while supporting patient access and scaling of manufacturing processes



### **Advancements in Regenerative Medicine Technologies**

CIRM supports research to improve regenerative medicine technologies ensuring the continuous evolution of the field



**Realization of  
CIRM's mission**



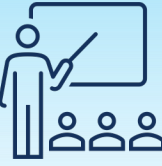
## New ReMIND\* Program with Neuropsychiatric Focus

**\$110M** approved to tackle complex **neuropsychiatric diseases** including **substance use disorders** in 2024-2028

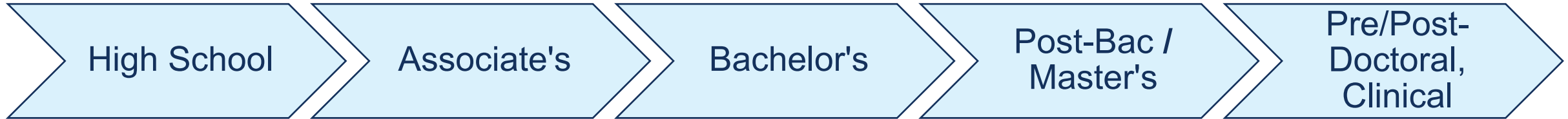
Opportunity	# Awards	Award Size
<b>ReMIND-L</b> Large collaborative projects	6	Up to \$10M
<b>ReMIND-X</b> Exploratory high-impact projects	12	Up to \$1M



\***ReMIND**: Research Using Multidisciplinary, Innovative Approaches in Neuro Diseases



## CIRM's Education Programs

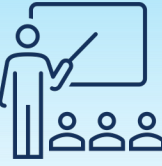


**SPARK**  
748 trained  
(11 summers)  
**+ 345 by 2026**

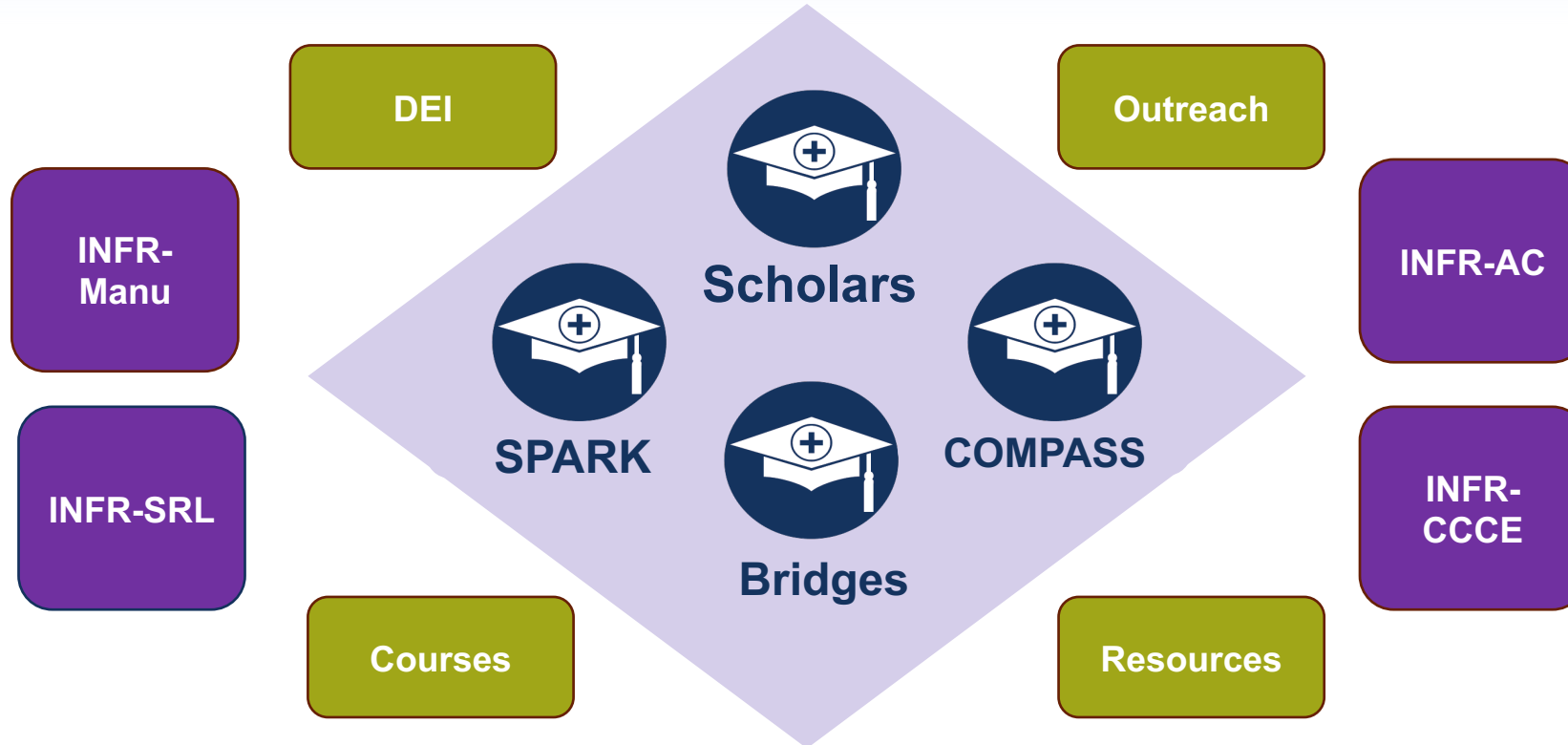
**Bridges**  
2014 trained/training  
(14 years)  
**+ 450 by 2026**

**Scholars**  
1204 trained/training  
(11 years)  
**+ 300 by 2026**

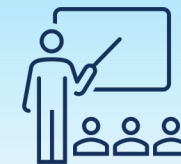
**COMPASS**  
127 training  
(1 Year)  
**+ 300 by 2027**



## Education Network Strategy | Overview



- **Leveraging** CIRM's investments in Education and Infrastructure Programs
- Building **partnerships** across institutions
- Building **mechanisms to share** educational resources and knowledge



## How Our Training Programs are Making an Impact

EDUC Program  
Trainee



Diverse Industry  
Positions



Mentorship of Current EDUC  
Trainees

**Keau Wong, MBT**



**CSU San Marcos  
Bridges 2011**  
Director, Bioscience Workforce  
Dev. Hub  
MiraCosta College

**Manuel Santana**



**Pasadena City College  
Bridges 2020**  
Adjunct Faculty  
Pasadena City College

**Kathy Ivey, Ph.D**



**Gladstone Institutes  
Scholar 2009**  
VP, Gene Therapy Research  
Tenaya Therapeutics

**Brian Fury, M.S**



**CSU Sacramento  
Bridges 2010**  
Director, GMP Facility  
UC Davis



## CIRM De-Risks Programs for Industry Investment

**CIRM-funded projects have attracted at least \$24.7 Billion in industry investments.**

- CIRM funding of academic projects has enabled the spinout of at least 50 companies



Neurona Therapeutics

## Highlighted Recent Partnering Events

- **Neurona Therapeutics**, a CIRM-funded California company developing regenerative medicines for neurological disorders, raised \$120 million in crossover financing in (Feb 2024)
- A CIRM-funded gene therapy for Cystinosis developed by Dr. Stephanie Cherqui at **UCSD** was acquired by Novartis for \$87.5 million (May 2023)



Jordan Janz and Dr. Cherqui



## CIRM De-Risks Therapy Development

### Cell Therapy for Treatment of Epilepsy



#### **5 Basic Bio or DISC Awards**

Development of protocols for derivation, characterization, selection, and production of interneuron precursors

#### **1 TRAN Award**

Development of inhibitory neurons as a therapeutic for focal epilepsy

#### **1 CLIN2 Award**

Phase 1/2 trial for a neural cell therapy for treatment of epilepsy

## 6 CIRM Awards to UCSF & Neuron Therapeutics (2007-2022)



Cory Nicholas

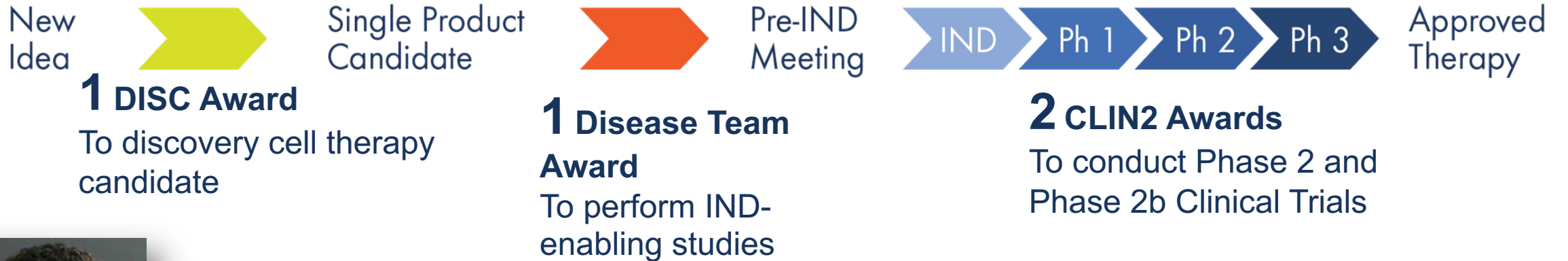




## CIRM Funding Accelerates Therapies to the Clinic

CIRM's Funding Model progresses therapies from initial discovery through clinical trials

### Cell Therapy for Treatment of Retinitis Pigmentosa



Henry Klassen

**4 CIRM Awards to UC Irvine & jCyte (2010-2019)**



## In Utero Stem Cell Therapy for Spina Bifida



**1 PC1 Award**  
To conduct preclinical development

**1 CLIN1 Award**  
To enable IND filing

**2 CLIN2 Awards**  
To conduct Phase 1 and Phase 2 Clinical Trials

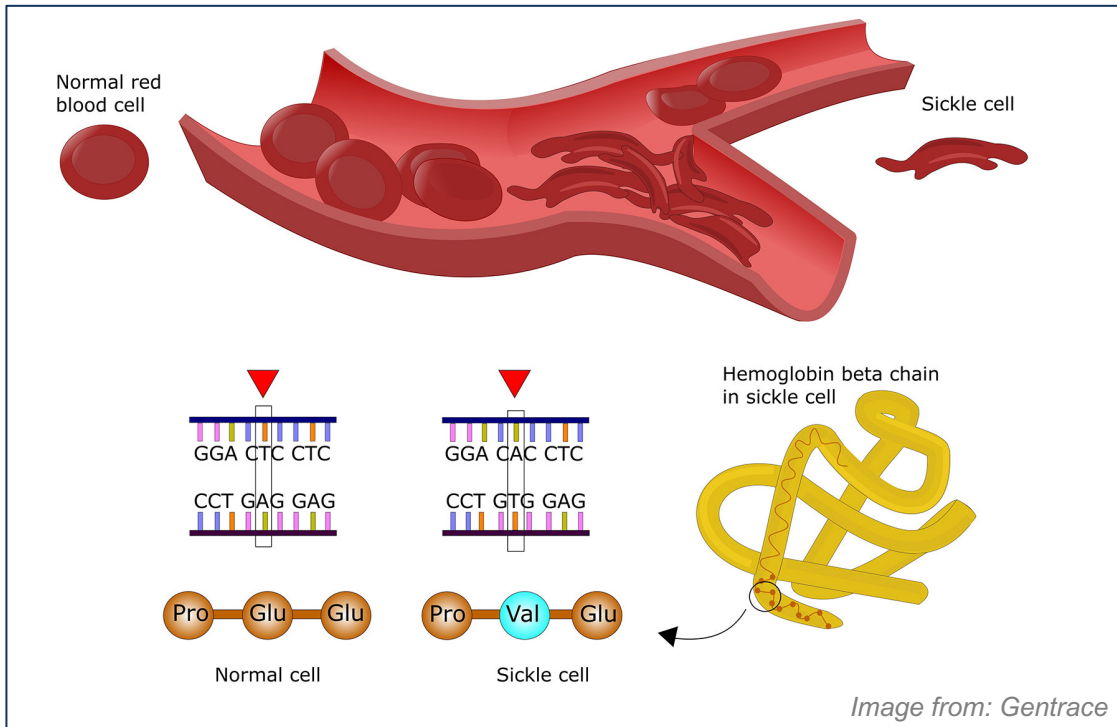


Dr. Diana Farmer and patients

**4 CIRM Awards to UC Davis (2015-Current)**



## Cure Sickle Cell Disease NHLBI-CIRM Partnership












## Sickle Cell Disease

- Caused by a point mutation in beta hemoglobin gene on Chromosome 11
- Affects ~100,000 in the U.S. & millions worldwide
  - 30% growth in the number of globally affected by 2050
- Average U.S. lifespan : ~40 years
- Complications: Debilitating pain, organ damage, strokes and hospitalizations



## CIRM Cure Sickle Cell Disease Programs

PI & Institution	Ex Vivo Cell-Gene Therapy Approach	Stage		
		Pre-Clinical	Early-Stage Clinical (Phase 1 and 2)	Late-Stage Clinical (Phase 3)
<p><b>Matthew Porteus</b> <i>Stanford University</i></p>	<ul style="list-style-type: none"> <li>Gene correction of mutation</li> <li>AAV6 delivery</li> <li>CRISPR/Cas9</li> </ul>		<p><i>Graphite Bio</i> <b>Phase 1</b></p> 	
<p><b>Donald Kohn</b> <i>UCLA</i></p>	<ul style="list-style-type: none"> <li>Genetic modification</li> <li>Lentiviral delivery</li> <li>Anti-sickling globin</li> </ul>		<p><b>Phase 1</b> </p> 	
<p><b>Mark Walters</b> <i>UCSF Benioff Children's Oakland</i></p>	<ul style="list-style-type: none"> <li>Gene correction of mutation</li> <li>Non-viral delivery</li> <li>CRISPR/Cas9</li> </ul>		<p><i>CIRM –NIH partnership</i> <b>Phase 1</b></p> 	
<p><b>David Williams</b> <i>Boston Children's Hospital</i></p>	<ul style="list-style-type: none"> <li>Fetal hemoglobin prevents sickling but is silenced postnatally</li> <li>shRNA to BCL11a (repressor of fetal hemoglobin expression)</li> </ul>		<p><i>CIRM –NIH partnership</i> <b>Phase 1/2</b></p> 	



## World-Class Cell and Gene Therapy Clinical Infrastructure



**200+** CLINICAL TRIALS

**1000+** PARTICIPANTS

**40+** DISEASE AREAS



Alpha Clinic



UC San Diego  
SANFORD STEM CELL INSTITUTE  
Sanford Stem Cell Clinical Center



USC+CHLA  
Alpha Clinic



## Overcoming Manufacturing Bottlenecks

**Enhance and interconnect academic and industry manufacturing resources to accelerate and de-risk development and delivery of cell & gene therapies**

### CIRM-Funded Manufacturing Network



### Industry Resource Partner Network



### Manufacturing Network Steering Committee

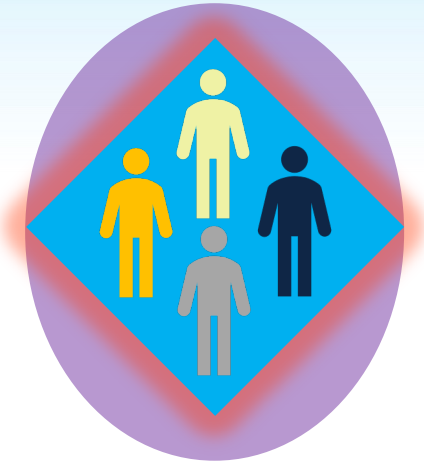


## Expanding Support Services for Patients

CIRM's New Patient Support Program (PSP) Administers



A Dedicated Patient Assistance Fund (PAF) Currently \$15.6M



- ICOC Consideration March 2024

- CIRM royalty revenues dedicated to this fund

**1** INFORMATIONAL

- Maintain a Patient Support (call) Center to direct patients to clinical trials
- Coordinate with trial sponsors to authorize participation

**2** LOGISTICAL COORDINATION

- Manage referrals to treatment centers
- Coordinate visits on behalf of patients

**3** FINANCIAL

- Administer PAF funds to cover patient costs
- Ensure non-duplication of costs or expenses

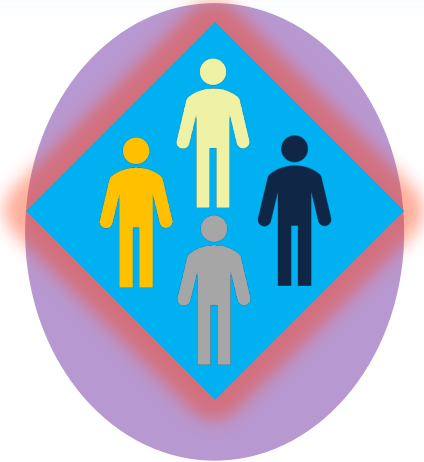


## Community Care Centers of Excellence Expand CIRM's Reach to Under-Represented Communities

Community Care  
Centers of Excellence  
Mandated by Prop 14



Concept Plan  
Approved January  
2024



- Mandated in Prop 14
- Intended to expand the geographic reach of treatment centers
- Intended to make treatments and cures that arise from institute-funded research more widely available to California patients,

- \$60.2 million approved
- Projected to support seven centers over five years
- Requires coordination with CIRM's Alpha Clinics, Patient Support Program and education programs
- Applications to open in Q1 or Q2 2024

- Strategic aim is to serve vulnerable and underserved populations
- Includes funding for community-based engagement of populations





## **New** - CLIN4: Getting CIRM-Funded Treatments Across the Finish Line



**CIRM Clinical Trial Programs**

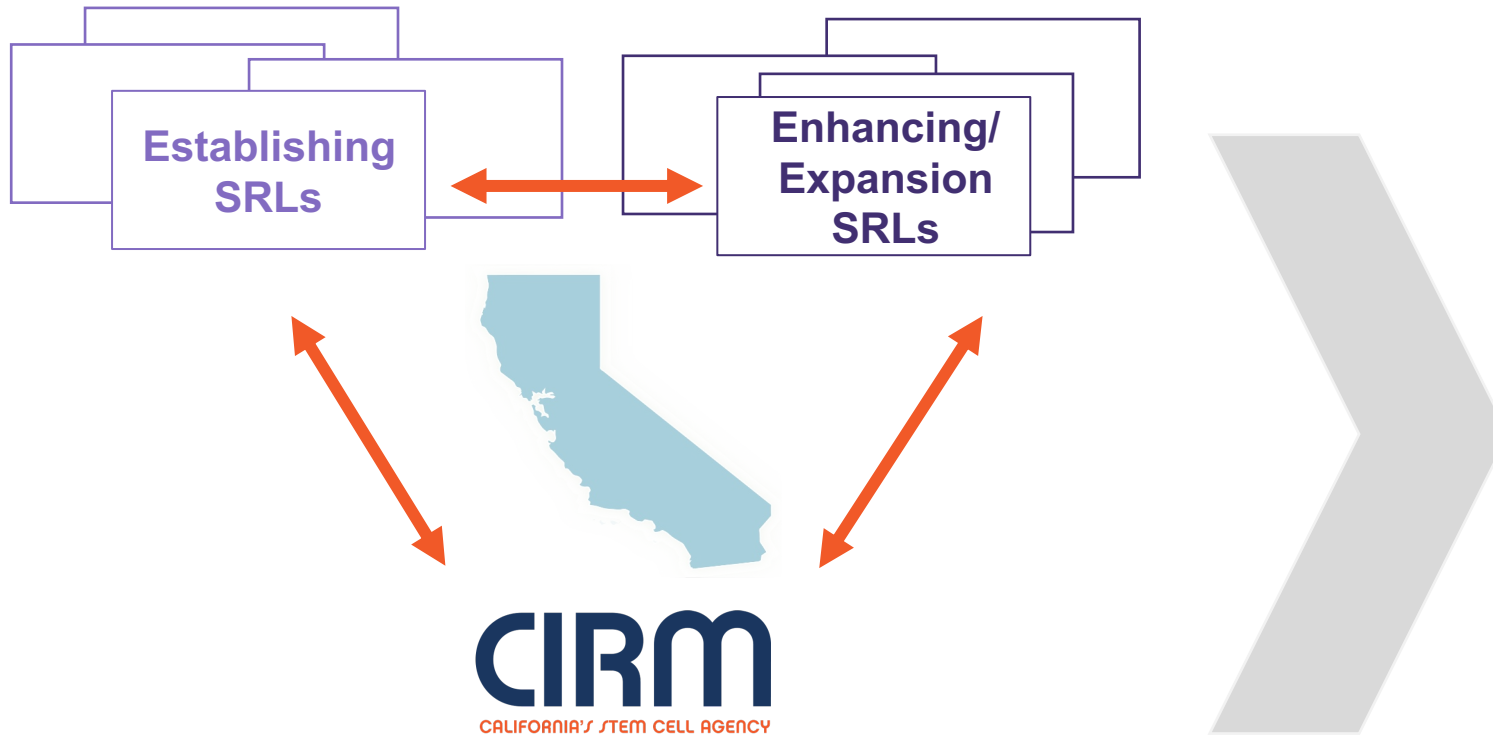


Activities Necessary to file a Biologics License Application:

- Manufacturing
- Pharmacoeconomic analysis
- Supply chain development



## Shared Resources Labs (SRLs): Increasing Access to Stem Cell-based Tools



- **Access** to stem cell models across CA
- **Advance** standards and reproducibility
- **Access** to educational opportunities
- **Develop** sustainable stem cell core infrastructure

**Thank You!**

Please visit us at:  
[www.cirm.ca.gov](http://www.cirm.ca.gov)

