Real Life™

President's Report

Annual Update on CIRM Strategic Plan

Maria T. Millan, MD
President & CEO
ICOC Meeting
March 28, 2023

CIRM
CALIFORNIA'S STEM CELL AGENCY

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CALIFORNIA'S STEM CELL AGENCY

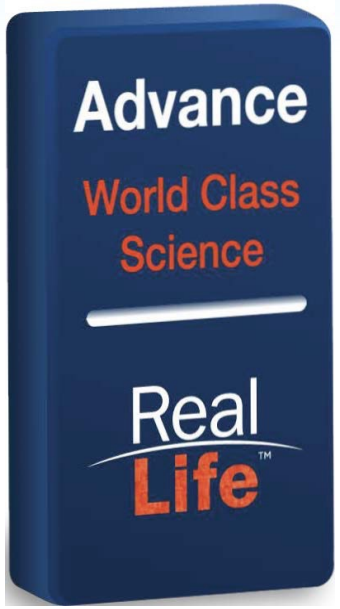
Real Life™

OUR MISSION

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

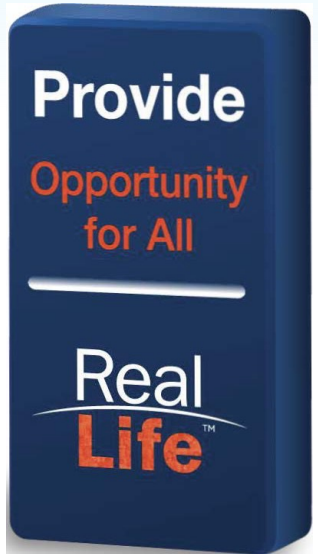
2022 Progress: Year 1 of 5-Year Strategy

Strategic Plan approved by ICOC December 2021



- Develop shared resources
- Build knowledge networks

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



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

Principles of Diversity Equity and Inclusion embedded within and across strategic goals

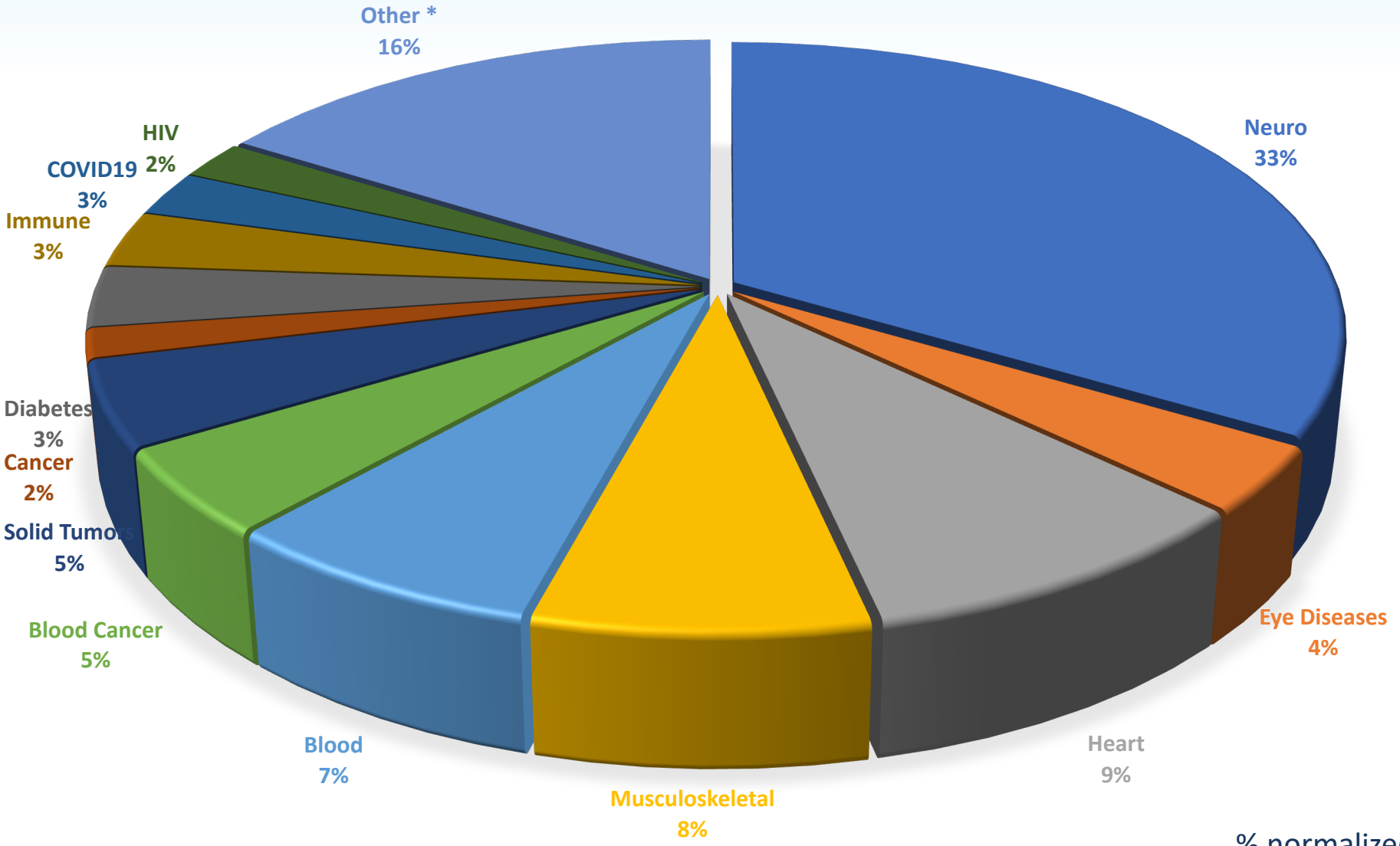
Advancing World Class Science through CIRM Funding

Historical (Prop 71+Prop 14) = $3.8B

**Not including the DISCO awards recommended for funding*

Data as of: Mar 2023

Research & Development Portfolio by Disease Area



Data as of: Dec 2022
% normalized to total R&D awards

Current Regenerative Medicine Landscape

CIRM Clinical Portfolio:

- **3 Active** Phase 2/ Phase 3 cell therapy programs (jCyte (Eye), Medeor (Kidney), Angiocrine (Vascular))
- **1 Completed** Phase 3 trial for ALS (Brainstorm) to be brought to FDA Advisory Committee
- **4 Active** Phase 2 *ex vivo* gene therapy; All in Rare Disease and * Pivotal (Rocket*, Kohn-UCLA, Cowan-UCSF, Williams- BCH*)
- **12** expedited designations (RMAT or Breakthrough)
- **3** Rare pediatric Disease Designations

27 FDA Approved Cell and Gene Therapy including :

2017 Gene therapy for Blinding Eye Disease (Spark, Roche)
First CAR T approvals (Kymriah (Novartis) & Yescarta(Kite))
2019 Gene therapy for Spinal Muscular Atrophy (Avexis, Novartis)
2022 5 FDA Approvals
Cell Gene therapy for Cerebral Adrenoleukodystrophy (Bluebird Bio)
CAR-T for relapsing Multiple Myeloma (Janssen)
AAV gene therapy for Hemophilia B (CSL Bhering, Uniqure)
Cell gene therapy for Beta thalassemia (BlueBird Bio)
Viral gene delivery IFN alpha -2b for Bladder CA (Ferring)

For full list : https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products

Role of CIRM funding in the current market

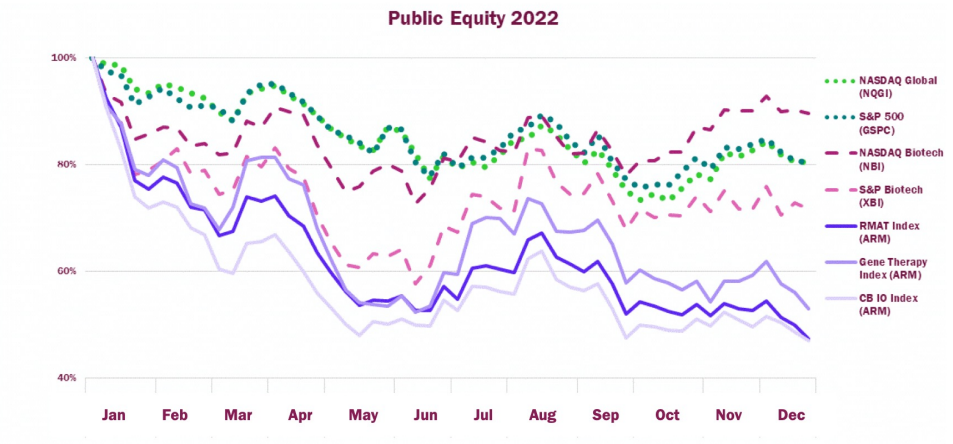
$24.2B Industry investment in CIRM programs to-date

CIRM programs achieved **$1.6B partnerships in 2022-2023 (f'ytd)**:

Capricor & Nippon Shinyaku Partnership - $735M
Aspen Neuroscience Series B - $147.5M
Vertex Acquisition of Viacyte - $315M
Rocket Pharma Public Offering - $108.2M
Juvena Series A Financing - $41M
Tenaya Public Offering - $75M
Jasper Therapeutics Public Offering - $103.5M
Calidi Bio SPAC Merger - $82M

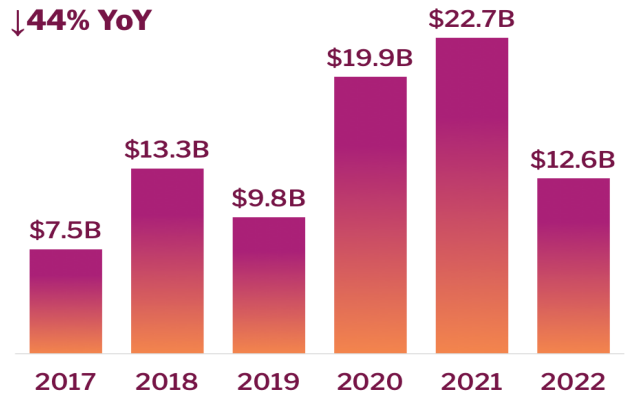
- The cost of capital is high and this has reflected a drop in investment in this fragile market & recent banking crisis
- CIRM provides a reliable source of funding for high-risk but high reward programs in this challenging market

Market Factors Depress Public Equity Performance



Investments "return to normal"

$12.6B raised in 2022



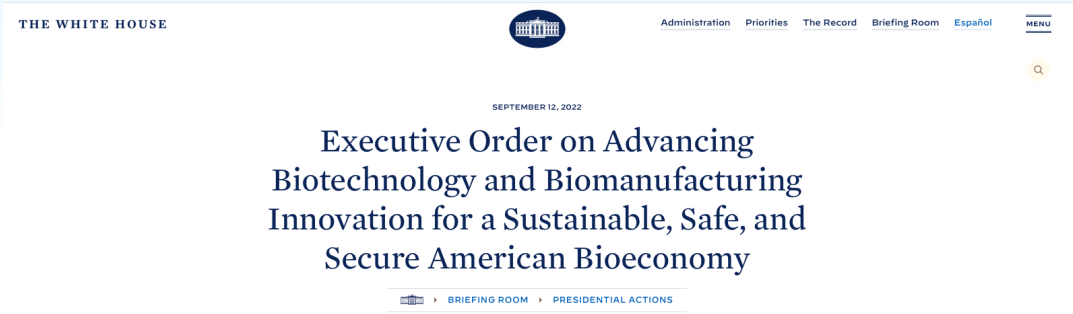
1. Includes upfront payments; excludes milestones, M&A, academic or government grants

Alliance for Regenerative Medicine, State of the Industry Briefing, Jan. 2023

Advancing therapies towards Commercialization

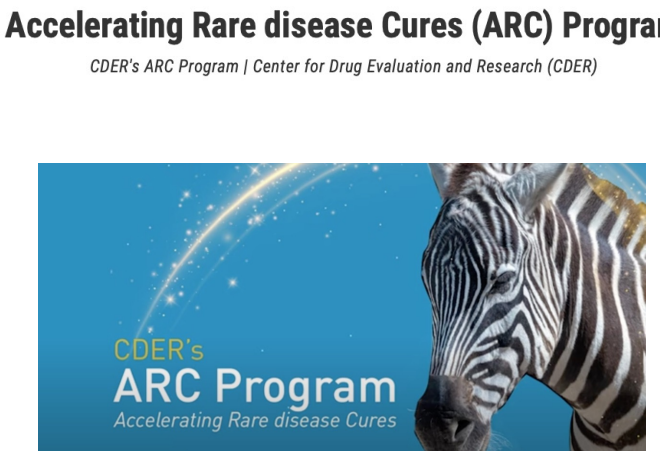
Manufacturing is a major hurdle to bringing promising therapies to patients

- Launch of the CIRM manufacturing network seeks to address a major hurdle while building a workforce
- Aligns with broader efforts (including Executive Order)



CIRM's efforts in rare disease presents an opportunity for collaborative efforts

- **50% of CIRM's active CLIN portfolio in rare disease**
- Partnership with Bespoke Gene Therapy Network (FNIH) & with NHLBI Cure Sickle Cell Disease supports consortia approach
- CIRM provides support in bridging the gap between academic efforts to industry/commercial setting
- Aligns with FDA efforts



Developing a Roadmap for Access and Affordability

2022 Events: *(Update from Sean Turbeville today)*

- Recruitment and Onboarding of Medical Affairs and Policy Team
- Team working with AAWG to shape Roadmap
- Patient Support Program RFA in final development *(Concept Amendment for ICOC consideration today)*
- Listening Sessions to inform Prop 14 Community Care Centers of Excellence Concept Proposal

US: The CGT wave arrives, to the benefit of patients

Up to 14 regulatory decisions expected

The FDA is evolving, by design, in order to keep pace

- Reorganization of OTAT to OTP 'super office'
- Filling current vacancies
- PDUFA VII adding 100 new reviewers in next five years
- CMC: PDUFA readiness pilot & potency assay workshop

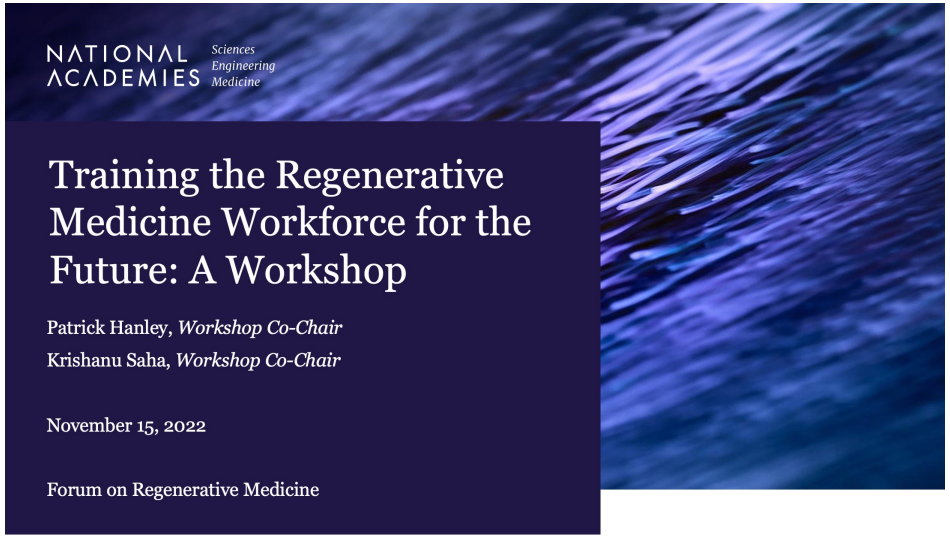
Questions linger about US payor readiness

- We need to modernize payment systems across Medicaid, Medicare and private insurers and expedite access to CGTs

Source: Alliance for Regenerative Medicine, State of the Industry Briefing, Jan. 2023

Building a Diverse and Skilled Workforce

- The field of regenerative medicine is growing rapidly; the demand for skilled workers is also increasing
- CIRM is designing approaches to integrate our EDUC and INFR programs for specialized training and on-ramps for underserved and underrepresented communities.
  - science, manufacturing, clinical research, regulatory and related fields.



Cross-cutting strategies: Open science and DEI

2022 Activities:

- DEI requirements included in CIRM grant applications & rubric and process implemented for GWG review
- DEI metrics are under development for tracking and management
- Data sharing and management guidelines have been implemented for grant applicants and post-award advisory being developed
- Board approval of the Shared Resources Labs Concept
- Convened a CNS Consortium Meeting

Nature 2023

WORLD VIEW | 11 January 2023 | Correction 13 January 2023

Why NASA and federal agencies are declaring this the Year of Open Science

JANUARY 11, 2023

FACT SHEET: Biden-Harris Administration Announces New Actions to Advance Open and Equitable Research

OSTP › BRIEFING ROOM › PRESS RELEASES

Plans for The Remainder of FY 2022-23

- Develop Neuro strategy with the Board Task Force (Prop 14 dedicates $1.5B for Neuro)
- Roadmap for Access and Affordability (an area of focus for Prop 14)
- Complete listening sessions and identify scope and priorities for Community Care Centers of Excellence Concept Plan (specified in Prop 14)
- Complete Launch of the Alpha Clinics Network Expansion Program (per Prop 14)
- Funding for Manufacturing Network Phase I Awards (Address bottlenecks)
- Deliver 2023-2024 Fiscal Year Budget (to support the above activities)
- DEI an ongoing priority for program development and review process

New Team Members FY 2022-23

2022

September

Emily Reyes
Project Manager,
Medical Affairs & Policy

Marivel De La Torre
Project Manager,
Medical Affairs & Policy

October

Ben Cahu
Associate Director, IT

November

James Campanelli
Sr. Sci Officer II, Therapeutics

December

Elizabeth Noblin
Sr. Sci Officer I, Review

2023

February

Charlie Shaw
Sr. Sci Officer II,
Business Development

Scott Tocher
Sr. Director,
Board Governance

March

Rafael Aguirre-Sacasa
General Counsel

Janie Byrum
Sci Officer,
Scientific Programs

Chan Lek Tran
Sr. Sci Officer II,
Scientific Programs

***Mark Burton**
Front Desk*
Contractor

April

Koren Temple-Perry
Sr. Director,
Marketing and Comms

Gemma Domingo
Sr. Finance Officer,
Finance

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Questions & Discussion