



President's Report

Annual Update on CIRM Strategic Plan

Maria T. Millan, MD
President & CEO
ICOC Meeting
January 26, 2023



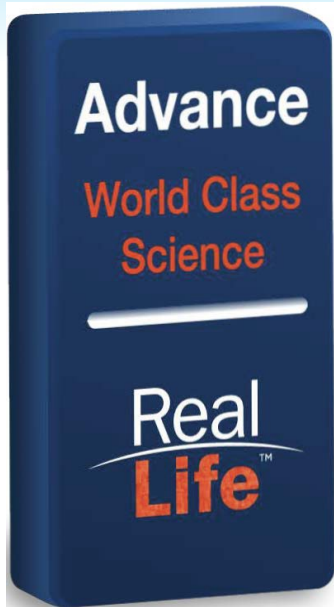
OUR MISSION

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

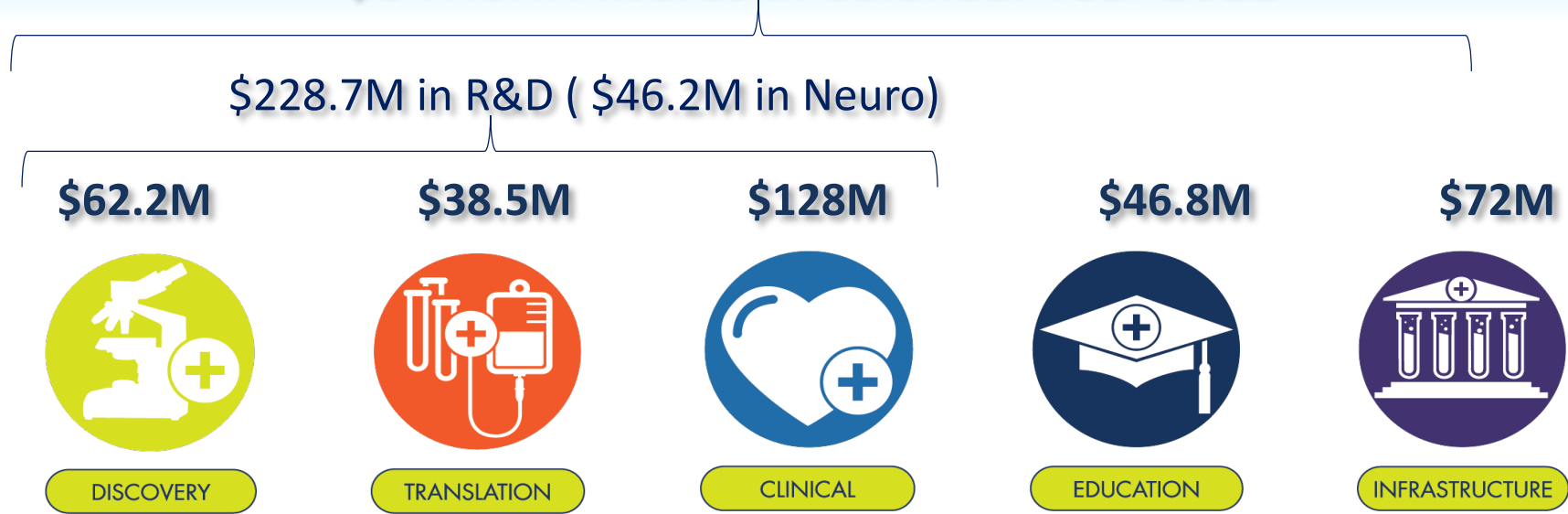


- Build a diverse and highly skilled workforce
- Deliver a roadmap for access and affordability

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

Advancing World Class Science through CIRM Funding

\$347.5M Awards in Calendar Year 2022



Available Funds for remainder Fiscal year (ends June 2023)

Discovery	Translation	Clinical	Education	Infrastructure
\$77.3M*	\$50M	\$69.5M*	\$0.95M	\$80M

**If newly recommended DISC and CLIN programs funded today by ARS*

Worldwide:

19 gene and gene- modified cell therapies (Blinding Eye Disease, Spinal Muscular Atrophy, CAR Ts)

18 RNA therapies are approved

59 non-genetically modified cell therapies are approved (includes cord blood, chondrocytes, mesenchymal, T cells)

Source: Pharmaprojects | Informa, July 2022 (presented by ASGCT)

2022 US FDA Approvals:

CAR T (Legend & Janssen) Multiple Myeloma

Gene Therapy Hemgenix (UniQure and CSL Behring) Hemophilia B

Gene Therapy (Adstiladrin (Ferring Pharma) Bladder Cancer

** For complete list of FDA approved cell and gene therapy products: <https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products>*

Status of Active CIRM Clinical Portfolio:

3 Phase 2/ Phase 3 cell therapy programs (jCyte (Eye), Medeor (Kidney), Angiocrine (Vascular))

4 Phase 2 *ex vivo* gene therapy; All in Rare Disease and * Pivotal (Rocket*, Kohn-UCLA, Cowan-UCSF, Williams- BCH*)

14 expedited designations (RMAT, Breakthrough or Fast Track)

2 Rare Pediatric Disease Designations

There are positive signals that regenerative medicine is now establishing itself as a field with significant industry involvement

The cost of capital and investment is high and this has reflected a drop in investment

CIRM provides a reliable source of funding for high-risk but high reward programs in this challenging market

\$24B Industry investment in CIRM programs to-date

CIRM programs achieved \$1.4B partnerships in 2022:

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

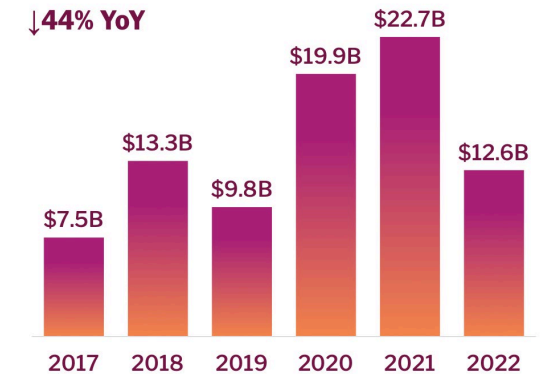
Market Factors Depress Public Equity Performance



Investments “return to normal”

\$12.6B raised in 2022

↓44% YoY



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

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)

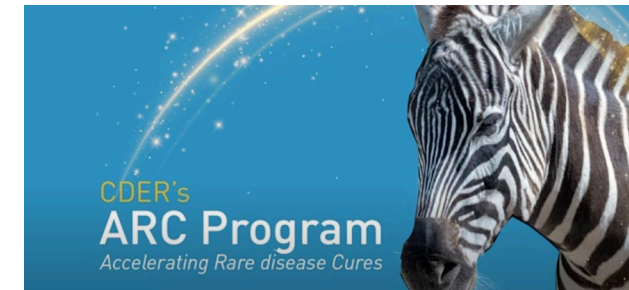
CIRM's efforts in rare disease presents an opportunity for collaborative efforts

- 49% of our 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



Accelerating Rare disease Cures (ARC) Program

CDER's ARC Program | Center for Drug Evaluation and Research (CDER)



2022 Activities:

- Recruited CIRM VP Medical Affairs and Policy and building internal team
- Working with AAWG to shape Roadmap
- Patient Support Program RFP developed to deploy Prop 14 Patient Assistance Fund
- 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

- The field of regenerative medicine is growing rapidly; the demand for skilled workers is also increasing
- The recent Executive Order on Advancing Biotechnology and Biomanufacturing Innovation for a Sustainable, Safe, and Secure American Bioeconomy highlights the timeliness of these discussions.
- CIRM's EDUC and INFR programs provides specialized training, opportunities and on-ramps for underserved and underrepresented communities and new models for the future workforce in science, manufacturing, clinical research, regulatory



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

WORLD VIEW | 11 January 2023 | Correction [13 January 2023](#)

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

Nature 2023

JANUARY 11, 2023

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

 › [OSTP](#) › [BRIEFING ROOM](#) › [PRESS RELEASES](#)

- 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)
- Launch CIRM's **Patient Support Program** (implement Prop 14 created patient assistance fund)
- Launch the **Alpha Clinics Network Expansion** (as specified in Prop 14)
- Deliver **2023-2024 Fiscal Year Budget** (to support the above activities)
- Launch the **Manufacturing Network** (a gating item to bringing therapies to the clinic)
- Launch **Shared Resources Labs Network** (5-year Strategic Goal)
- **DEI** an ongoing priority for program development and review process

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Questions & Discussion