

CIRM Funding Opportunities for Pre-Clinical & Clinical Development of Regenerative Medicine Therapies

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Freemind Non-Dilutive Funding Summit
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OUR MISSION

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



Proposition 71
2004

\$3B Bond
Funding



Proposition 14
2020

\$5.5B Bond
Funding

\$3.6B in total grants to date



DISCOVERY

\$1.1B



TRANSLATION

\$503M



CLINICAL

\$1.1B



EDUCATION

\$430M



INFRASTRUCTURE

\$489M

Prop 14 era

\$75M*

\$77M

\$147M*

\$190M

\$3M

2004

CIRM created by patient advocates and California stakeholders-Proposition 71

2020

Refunded under Proposition 14 - **\$5.5B** committed to CIRM mission

1100+

Cutting edge research & transformative programs funded

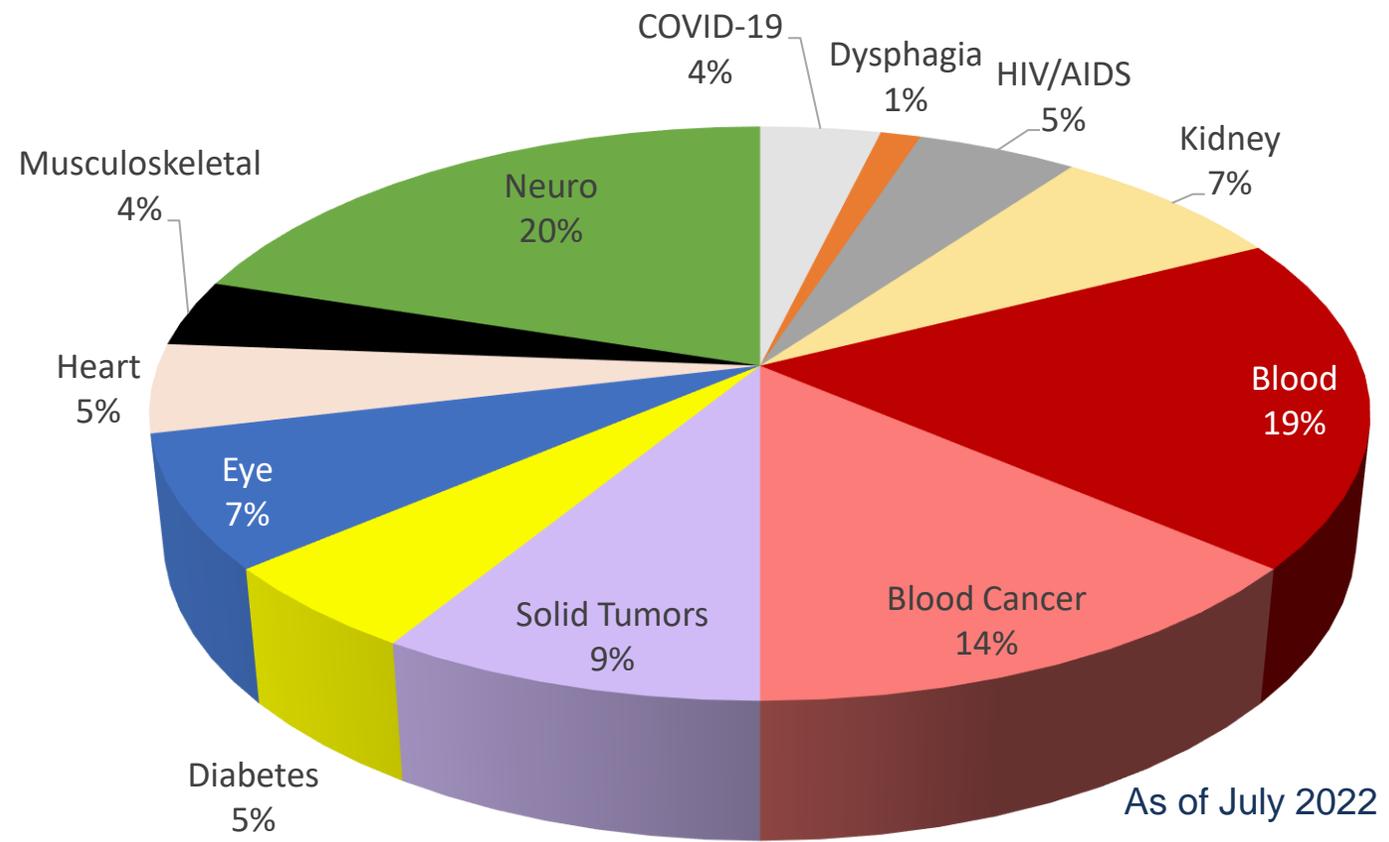
86 Clinical Trials, 3200+ Patients Enrolled

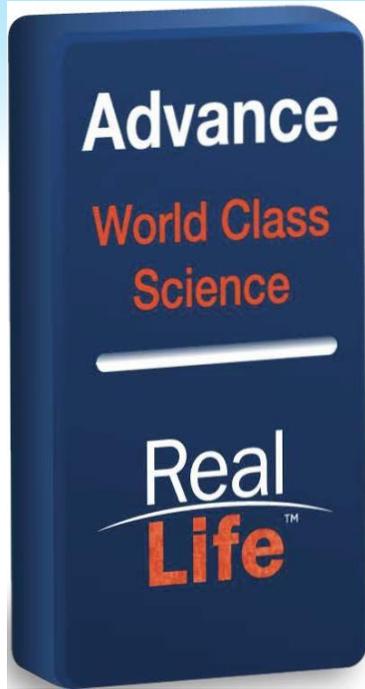
First in human, cell & gene medicine, some ready for final marketing approval

\$23B

CIRM-funded projects raised over \$23B in industry support

86 Clinical Trials funded through December 2022
across therapeutic areas





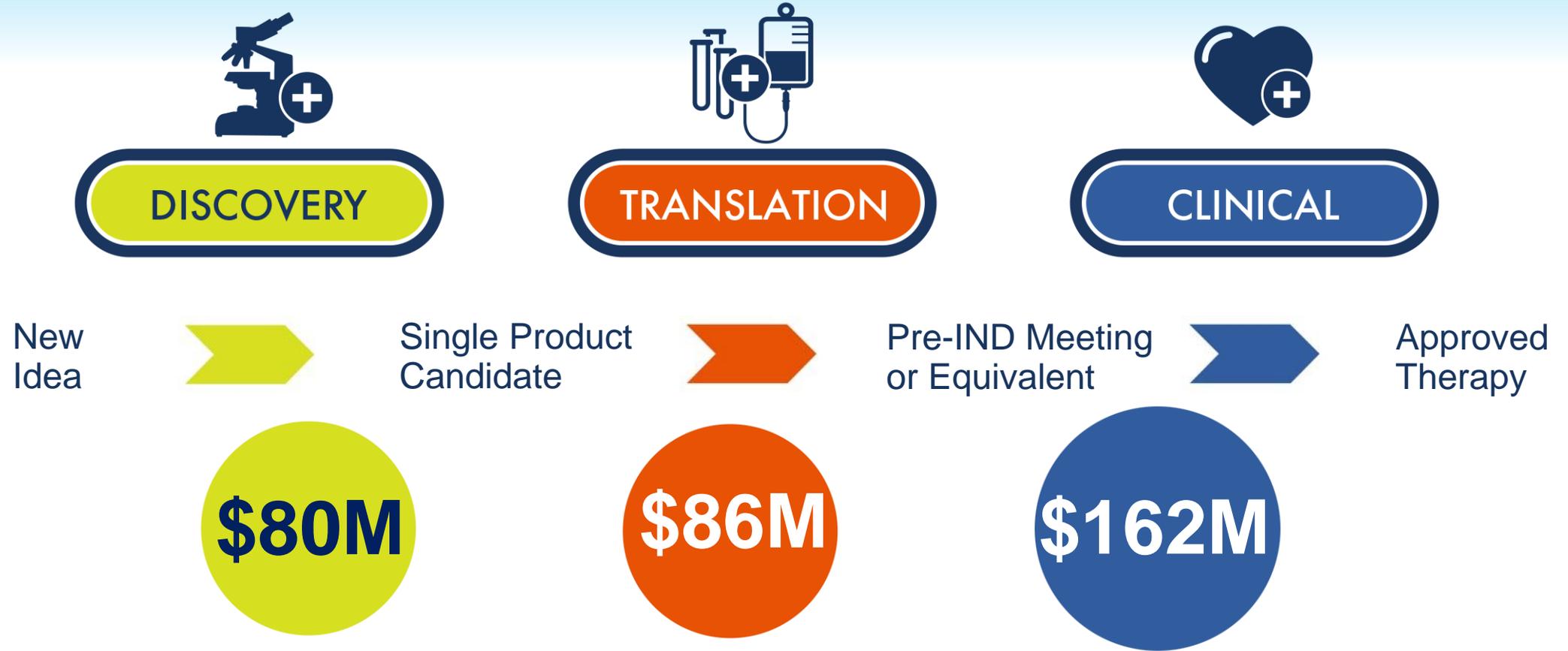
- Develop shared resources
- Build knowledge networks



- 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



FY 2021/22 Funding Allocation



DISCOVERY



TRANSLATION



CLINICAL

Foundational Knowledge



Single Product Candidate



Pre-IND Meeting or Equivalent



Approved Therapy



2/Year



2/Year



12/Year



Program	Scope	Award Outcome	Max Award Amount	Co-Funding	Max Duration (months)	Progression
DISC0 (Basic Biology)	Knowledge Gap	Basic Research	\$1M*	N/A	36	DISC2
DISC2 (Candidate Discovery)	Therapeutic	Therapeutic Candidate	\$1.5M*	N/A	36	TRAN1
	Technology	Technology Candidate	\$500K*	N/A	24	TRAN 2-4

*Indicates Direct Costs Max



Program	Project Readiness	Award Outcome	Max Award Amount	Co-Funding#	Max Duration (months)	Progression
TRAN Translational	Therapeutic Candidate	Pre-IND	\$4M*	20%	30	CLIN1
	Diagnostic Candidate	Pre-Sub	\$1.2M*	20%	24	
	Device Candidate	Pre-Sub	\$2M*	20%	24	
	Tool Candidate	Design Validation	\$1M*	20%	24	N/A

*Indicates Direct Costs Max

#Required for all for-profit applicants.



CLINICAL

Clinical Programs

Program	Project Readiness	Award Outcome	Max Award Amount	Co-Funding#	Max Duration (months)	Progression
CLIN1 (IND/IDE Enabling)	Pre-IND / Pre-Sub Completed	IND/IDE Clearance	\$4M-\$6M	20%	24	CLIN2
CLIN2 (Clinical Trials)	Active IND / IDE	Complete Dosing & Initial Analysis	\$8M-\$15M	30-40%	48	CLIN2

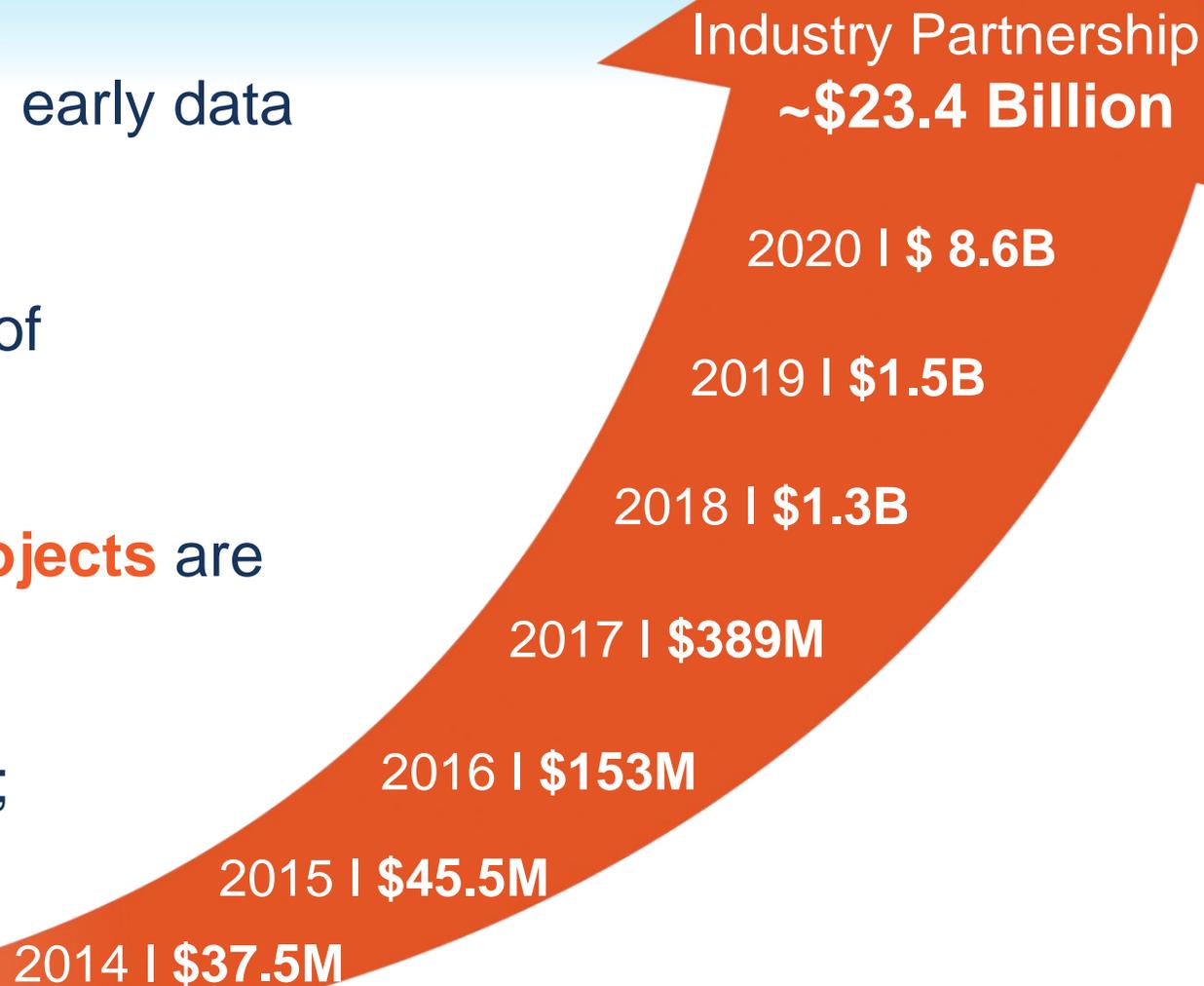
#Required for all for-profit applicants. Required for all applicants for phase 2 and later trials

CIRM nurtures and de-risks therapeutic development programs until they obtain early data to attract industry partnerships.

CIRM funding has enabled the spinout of **50 companies** from academia.

Over **50% of CIRM-funded clinical projects** are partnered with industry.

6 portfolio companies have gone public;
4 were acquired



Cell Therapy for Treatment of Retinitis Pigmentosa



Henry Klassen

4 CIRM Awards to UC Irvine & jCyte (2010-2019)
jCyte entered into \$252M Partnership with Santen Pharmaceutical (2020)

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

7 CIRM Awards to UCSF & Neurona Therapeutics (2007-2022)

Neurona Therapeutics has raised \$135M in venture financing to date



Cory Nicholas

CIRM Provides Resources to Accelerate Projects

CLIN and TRAN projects participate in Advisory Panels which are confidential, non-binding, with the goal to accelerate projects to achieve operational milestones.



Alpha Clinics Network: Making Clinical Trials Accessible to Many



- \$40M investment in patient-centered care and training (additional \$80M budgeted for 2023)
- Successfully accelerating cell and gene-therapy clinical trials
- Over 105 trials supported since 2015
 - 82 from industry sponsors
 - 23 CIRM-funded industry and academic sponsors
- Over \$100M in industry-contracts

CIRM's Existing and Sharable Tools for Disease Research & Drug Discovery

World's largest (2600) human iPSC repository for research

- Repository managed by FUJIFILM Cellular Dynamics
- Diverse ancestries including African, Admixed American (i.e. Hispanic, Native American), East & South Asian, European
- Standardized consents, iPSC derivation and QC testing
- Pre-negotiated license agreement available for commercial use
- Available Soon: SNP genotyping data on almost entire collection and WGS on 300+ lines



USC

UCI

UCLA



UCSF



FUJIFILM



CIRM
CALIFORNIA'S STEM CELL AGENCY

**CIRM Human Induced Pluripotent
Stem Cell Repository**

6 Diseases Classes, 17 Diagnoses
Matched Controls for All Diseases

- Age-Related Macular Degeneration
- Alzheimer's Disease
- Autism Spectrum Disorder
- Cardiomyopathies
- Cerebral Palsy
- Diabetic Retinopathy
- Epilepsy
- Fatty Liver Diseases
- Hepatitis C
- Intellectual Disabilities
- Major Depressive Disorder
- Movement Disorders (ADCY5)
- Optic Nerve Hypoplasia
- Phelan-McDermid Syndrome
- Primary Open Angle Glaucoma
- Pulmonary Fibrosis

BROWSE NOW

Goal: To overcome manufacturing hurdles for the delivery of regenerative medicine therapies by building a public-private manufacturing partnership network

**CIRM-Funded
Academic GMP
Facility Network**



Industry Partners

- Manufacturing Services
- Resources
- Investment and Partnerships



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



INDUSTRY
ALLIANCE
PROGRAM

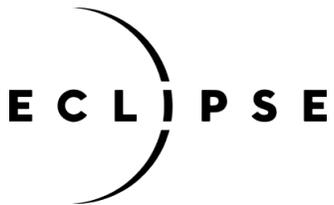
CIRM's Industry Alliance Program (IAP): A unique opportunity for the industry to partner with CIRM in accelerating clinical and preclinical stem cell, gene and regenerative medicine therapy programs to market.



PANACEA VENTURE
HEALTHCARE



BlueRock
Therapeutics



SYNTHEGO



khosla ventures

Clinically Compatible Stem Cell Lines



Manufacturing Resources





- **CIRM and NHLBI MOU to jointly fund industry and academic cell and gene programs for the Cure Sickle Cell Initiative**
- Leverages CIRM's processes and funding infrastructure
- American Society of Hematology setting up registry & data capacity

- **CIRM joined the Accelerating Medicines Partnership® Bespoke Gene Therapy Consortium (AMP® BGTC)**
- Consortium of the NIH, FDA and over 20 private sector partners, managed by FNIH
- Goal is to create a standard blueprint for accelerated development of AAV gene therapies for rare genetic diseases
- Eligible CIRM funded programs will be part of this Consortium
- CIRM sits on the Steering Committee



Thank You!

Please visit us at:
www.cirm.ca.gov

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