

CIRM's Evolution and Vision for the Future

Jonathan Thomas, PhD, JD
CIRM President & CEO
Citizens Financial Accountability
Oversight Committee (CFAOC)
December 2024

C I R M
CALIFORNIA INSTITUTE FOR
REGENERATIVE MEDICINE



Photo: UCLA Broad Stem Cell Research Center



Our Mission

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



Photo: Cal Poly Humboldt



Since 2004, CIRM has funded **\$3.8 billion*** in regenerative medicine research, infrastructure, and education programs, making California a hub for cell and gene therapy innovation.



Photo: Barbara Ries / UCSF

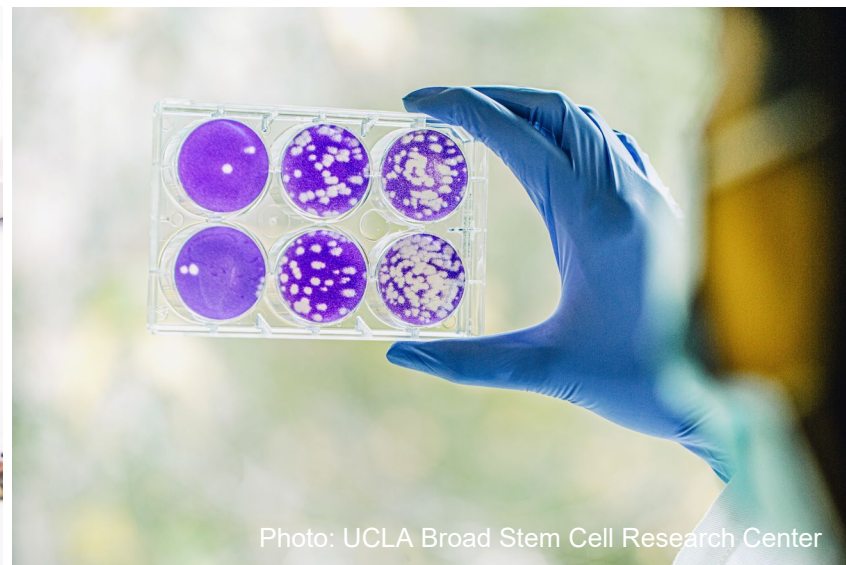


Photo: UCLA Broad Stem Cell Research Center



Photo: Cal State Fullerton

*As of June 30, 2024

Our Impact



85+

Diseases
and
conditions



9

Alpha Clinics
across the
state



4,300+

Interns,
trainees,
and fellows



50+

New
businesses



56K+

full-time
equivalent
jobs in CA*

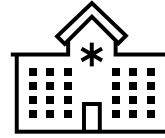
*As of 2019 Economic Impact Report

Prop 14-Era: Our 5-Year Strategic Plan



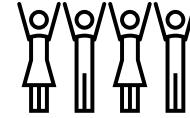
Advance World Class Science

- 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

Diversity, Equity, & Inclusion (DEI)

CIRM requires diverse representation in the projects it funds and promotes DEI values across our programs, including:

- In patient outreach and clinical trial recruitment
- In education and workforce development
- Providing access to diverse cell lines for research (iPSC Repository)
- Advancing our community outreach efforts across the state



Overview of Funding Programs & CIRM-Funded Research

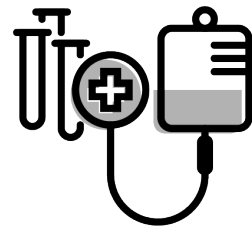
Pillar Programs and Investments



DISCOVERY

PROP 71/PROP 14
PROP 14 Only

\$1.2B
\$170M



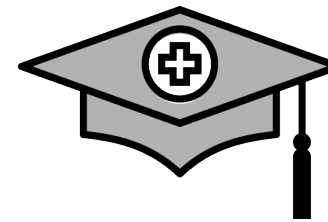
TRANSLATIONAL

\$656M
\$230M



CLINICAL

\$1.5B
\$500M



EDUCATION

\$439M
\$198M



INFRASTRUCTURE

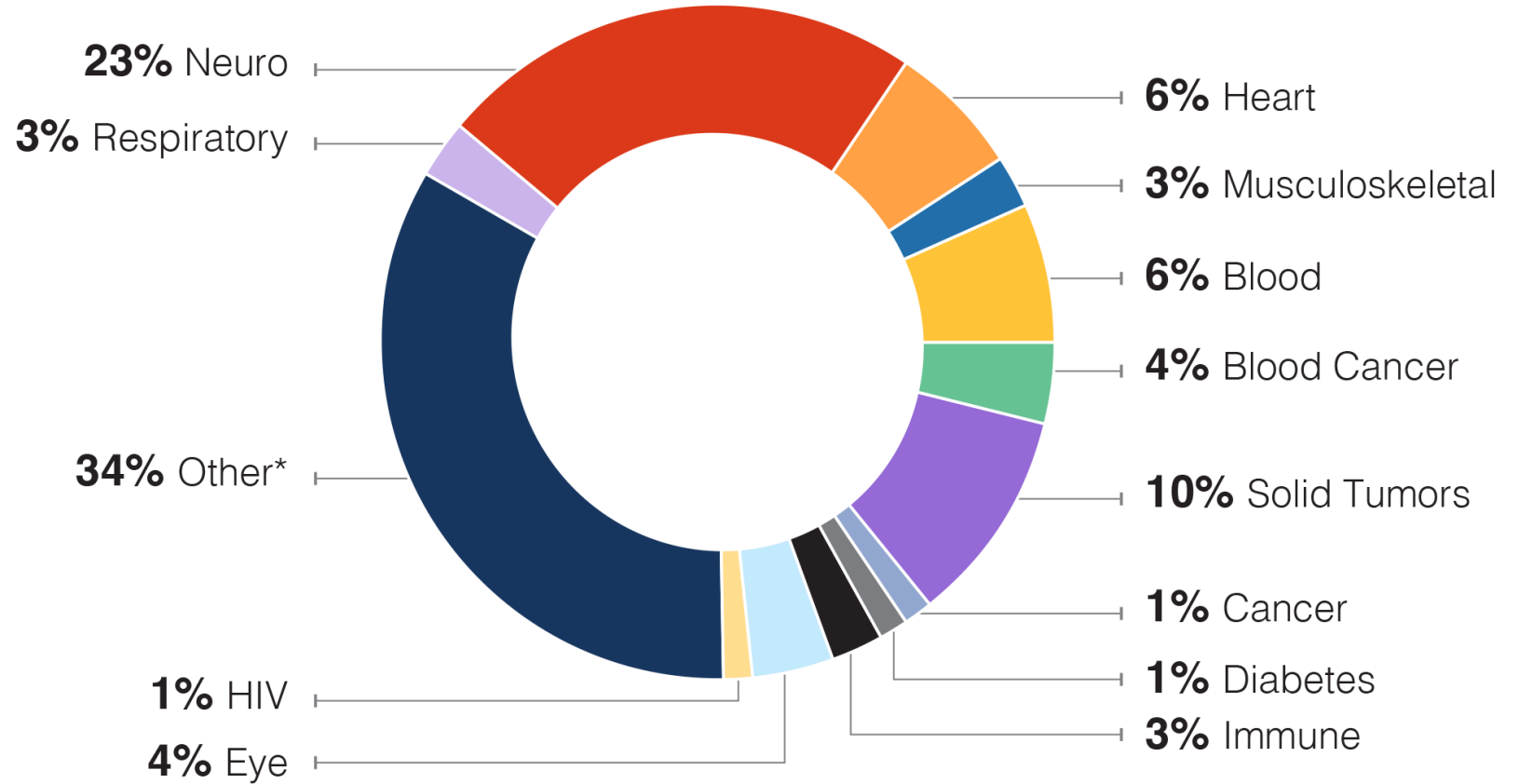
\$629M
\$140M



\$275M in the neuro space in Prop 14 era

Research and Development Portfolio*

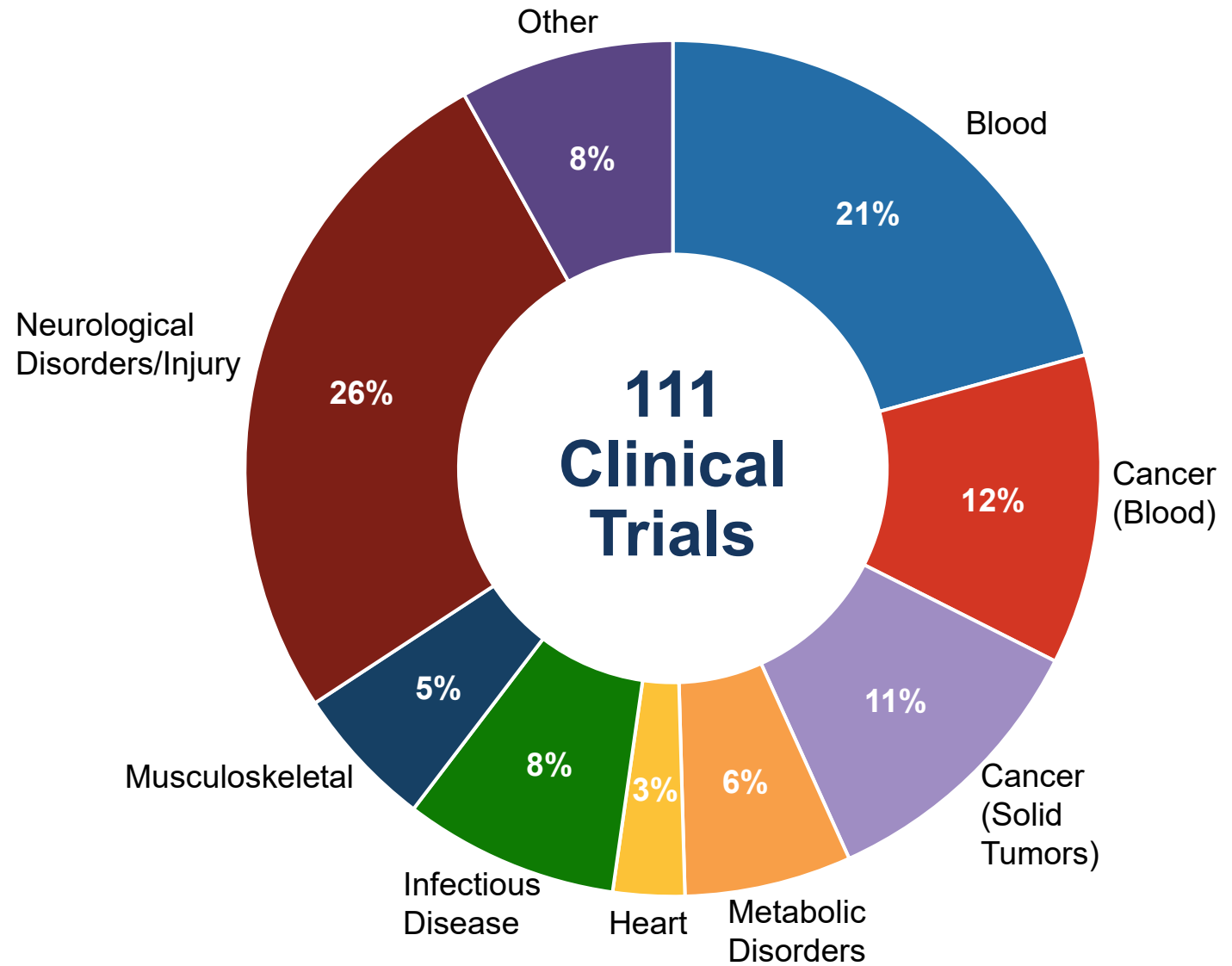
- Wide variety of diseases and disorders
- Cell and gene therapies, biologics, small molecules



* FY 2023/2024

Clinical Trial Portfolio

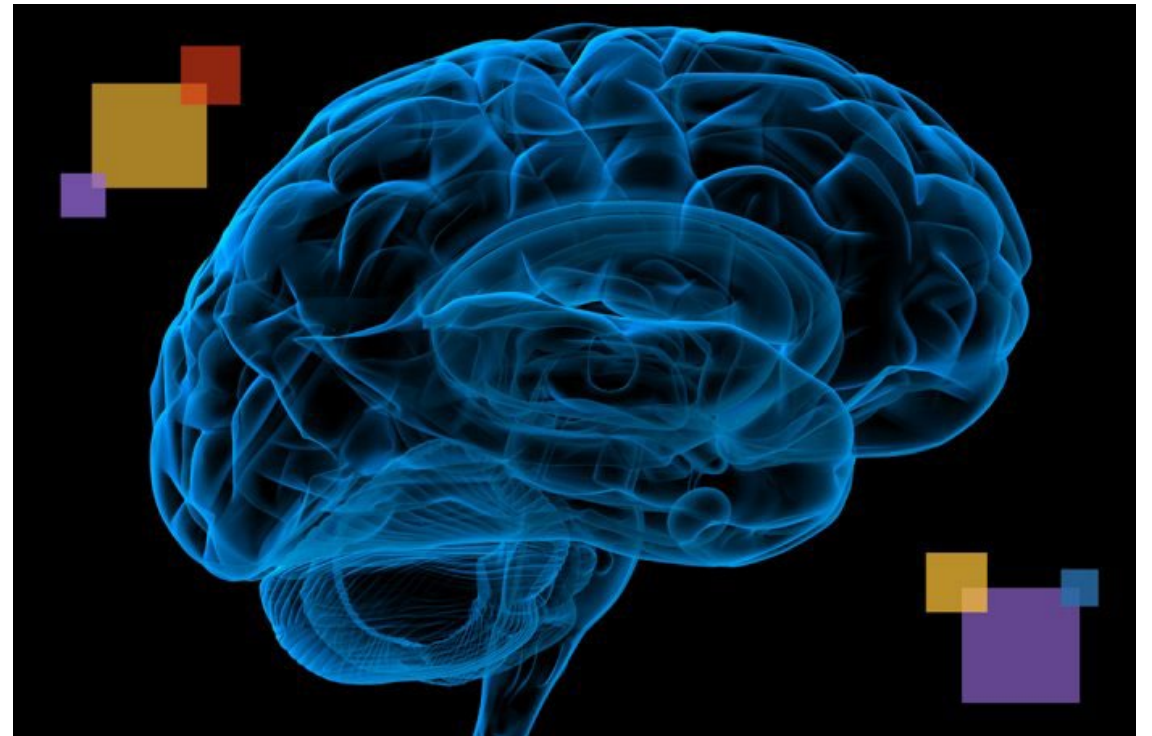
- 111 Clinical Trials funded*
- Wide variety of diseases and disorders
- Different approaches



*As of December 2024

\$1.5 Billion for Neuro Research

- **ReMIND: Research Using Multidisciplinary, Innovative Approaches in Neuro Diseases**
- Allocating at least **\$1.5 billion** for research and development of treatments for diseases affecting the brain and central nervous system
- **\$110 million** approved to tackle complex neuropsychiatric diseases including substance use disorders in 2024-2028
- **\$67.5 million** approved in Aug 2024 to support 5 projects in pilot ReMIND Program



Building a Diverse Regenerative Medicine Workforce in California

To date, CIRM's
education and training
programs have
supported **4,317+**
interns and fellows.



Training Programs: SPARK and COMPASS

SPARK



- 11 programs across state
- High School Students
- Summer Laboratory Internship
- End of Summer Conference

COMPASS



- 16 Programs across state
- Undergraduates – Sophomore/Junior
- 2-3 years support, courses + internship(s)
- Innovative Recruitment and Selection

Training Programs: Bridges and Scholars

Bridges



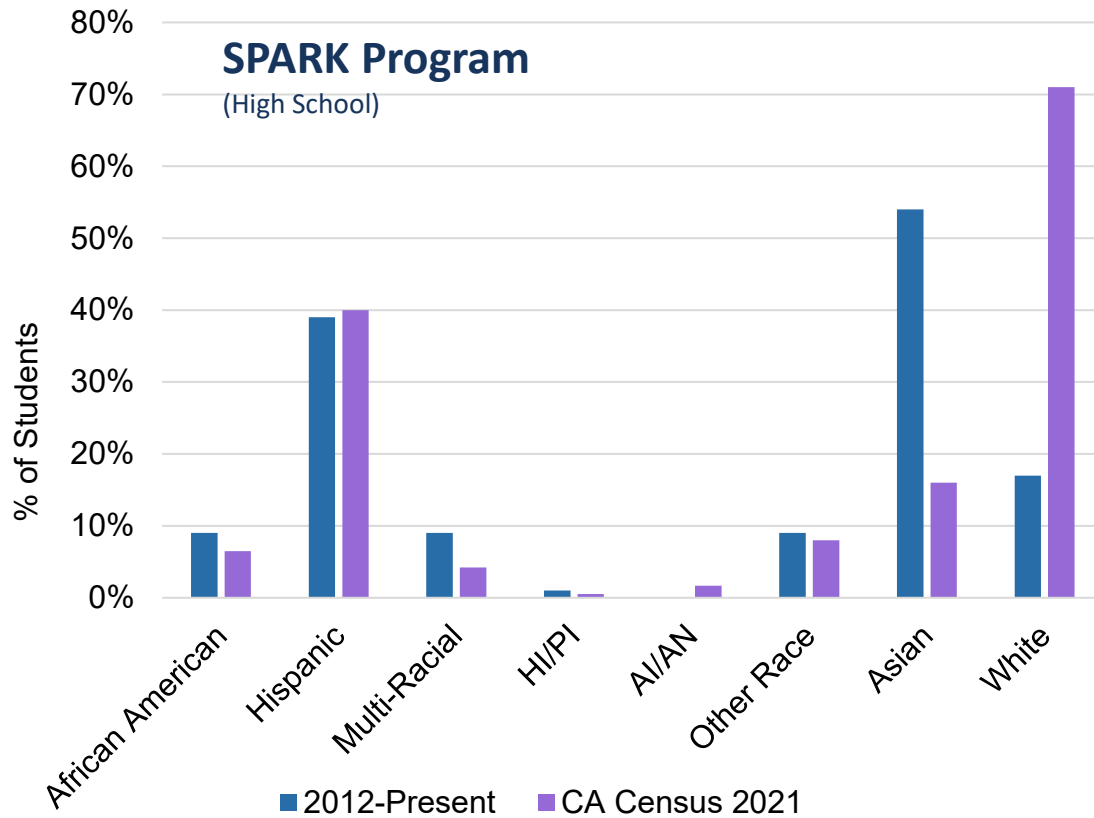
- 15 Programs at CSUs/Comm Colleges
- Late undergrad, Masters, or special certificate
- Specialized courses, activities and outreach
- Up to 12 months internships at host sites

CIRM Scholars



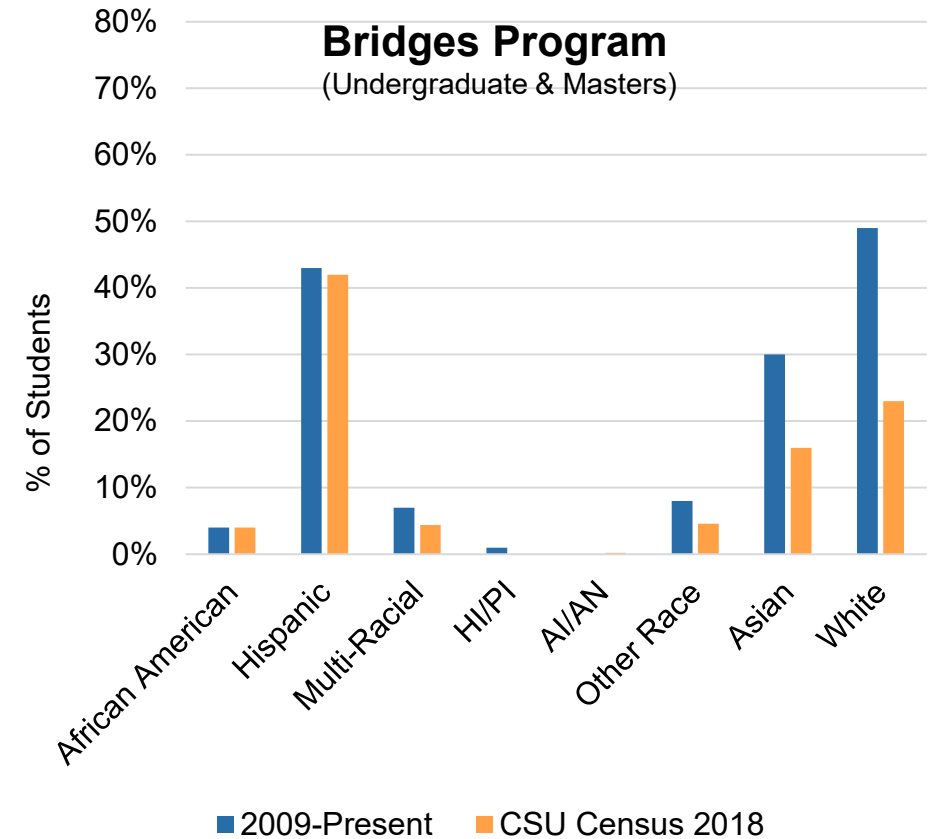
- 18 programs across state, 3rd RFA
- Predoc, postdoc, clinical Fellows
- Special Courses/Outreach
- 2-3 Year Fellowships

CIRM education programs aim to **broaden participation** of individuals representing the diversity of California including **those who may not have had opportunities** and are hindered by socioeconomic constraints.



N for Race = 454
N for Ethnicity = 307

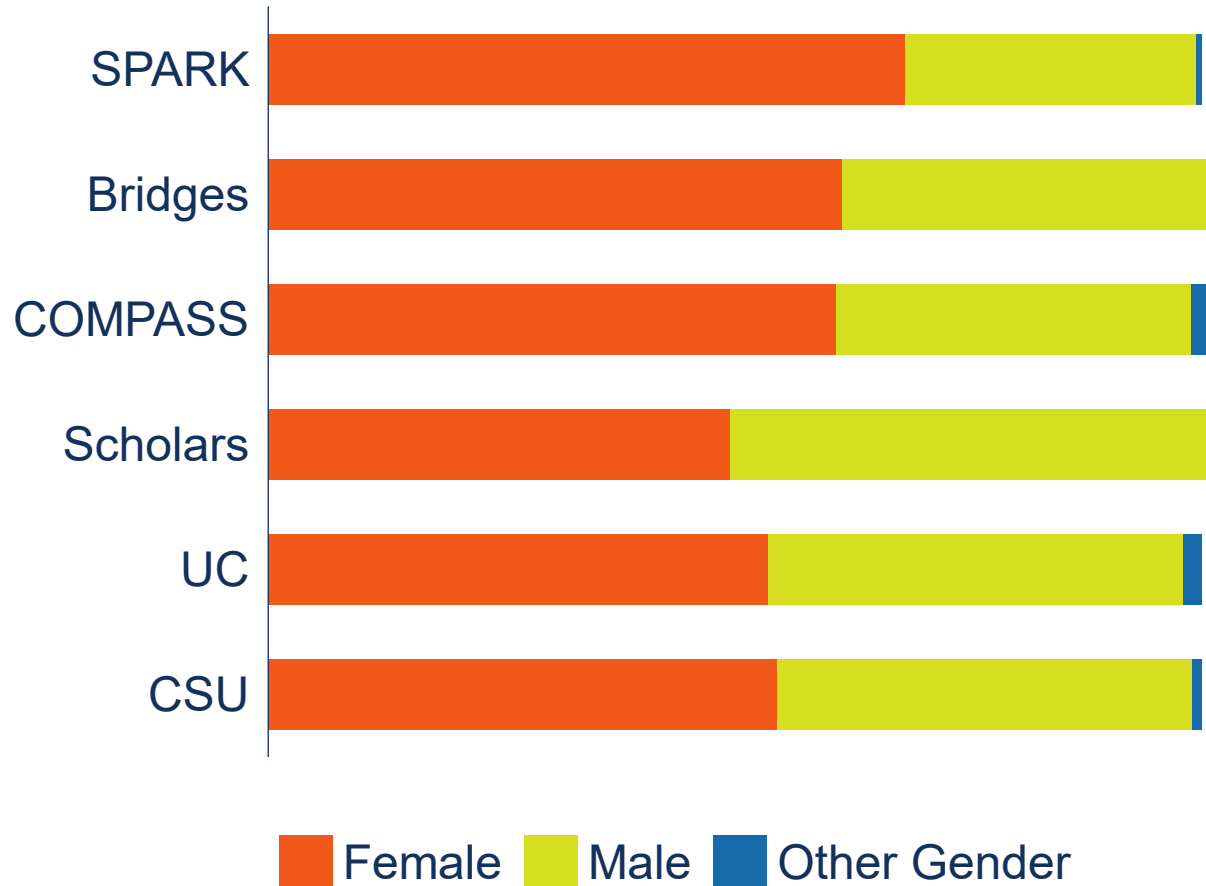
*includes Hispanic and other racial categories



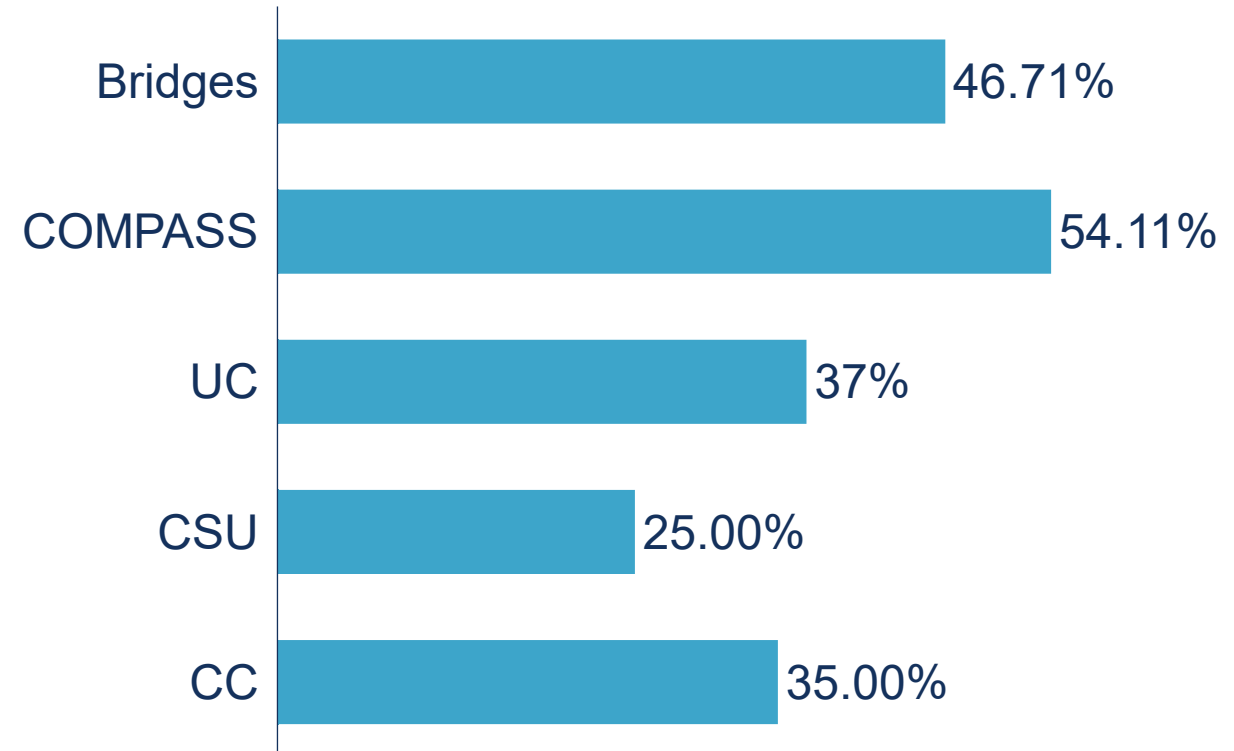
N for Race = 1587
N for Ethnicity = 994

Gender Identity and First-Generation Students

Gender Identity



First Gen Students



Bridges: N=957

COMPASS: N=134, 12 (8.2%) unreported

Commercialization of Cell and Gene Therapies

Alpha Clinics Network: Supporting World-Class Clinical Infrastructure



- Nine leading medical centers
- 250+ trials supported
- CIRM-funded and industry sponsors
- Over 2,000 patients treated
- Over \$100 million in industry contracts



Building a California Cell and Gene Therapy Manufacturing Network

Composed of 9 CIRM-funded academic Good Manufacturing Practice (GMP) facilities in California to help overcome manufacturing bottlenecks

Goals of the network:

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

Network Members



Patient Support Program: Addressing Access Barriers to Trials

- \$15.6 million patient assistance fund
- Promotes equal access to CIRM-funded clinical trial
- Addresses financial and logistical challenges faced by patients and their families



Photo: UCLA Broad Stem Cell Research Center

Community Care Centers of Excellence: Expand Access for Underrepresented Communities

- Enable broad awareness and access to regenerative medicine treatments for patients
- Expanding access to clinical trials, treatments, and CIRM education and training programs
- Expected to launch in 2025



Photo: Barbara Ries / UCSF

Forging Partnerships to Commercialize Cell and Gene Therapies

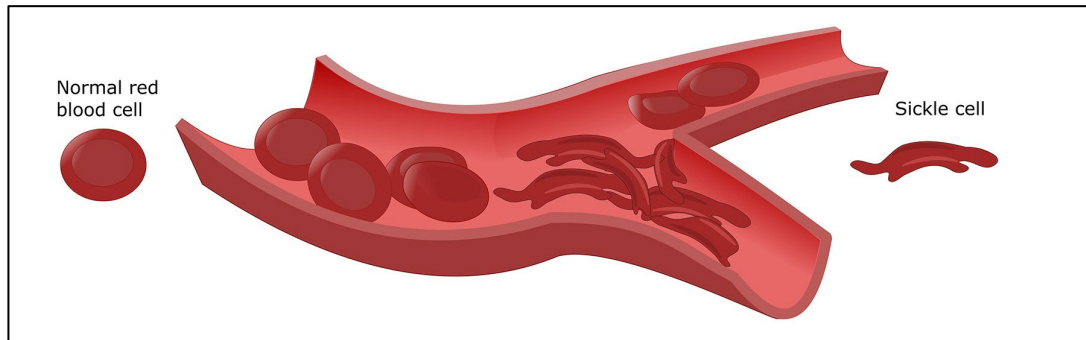


Image from: Gentrace

- Sickle Cell: Affects around 100,000 in the U.S. & millions worldwide
- Four CIRM-funded trials for sickle cell:
 - **Matthew Porteus**, *Stanford University*
 - **Donald Kohn**, *UCLA*
 - **Mark Walters**, *UCSF Benioff Children's Oakland*
 - **David Williams**, *Boston Children's Hospital*
- Part of a unique partnership with the National Heart, Lung and Blood Institutes (NHLBI) under the NIH's "Cure Sickle Cell" initiative

Looking Into the Future

Strategic Allocation Framework (SAF): A structured, data-driven effort designed to **prioritize resources and maximize the impact** of CIRM's \$3.86 billion left in funding

New Impact Goals

1. **Catalyze** the identification and validation of at least 4 novel targets and biomarkers, ensuring integration into preclinical or clinical research for diseases in California
2. **Accelerate** development and utilization of 5-8 technologies that demonstrate improvements in safety, efficacy, or quality of cell and gene therapies
3. **Advance** at least 4-7 rare disease projects to BLA
4. **Propel** 15-20 therapies targeting diseases affecting Californians to late-stage trials
5. **Ensure** that every BLA-ready program has a strategy for access and affordability
6. **Bolster** CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine

Thank You!

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