

**SOMETHING
BETTER
THAN HOPE**

Scientific Strategy Advisory Panel

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Portfolio Development and Review

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CIRM Investments: Five Funding Pillars



DISCOVERY



TRANSLATION



CLINICAL



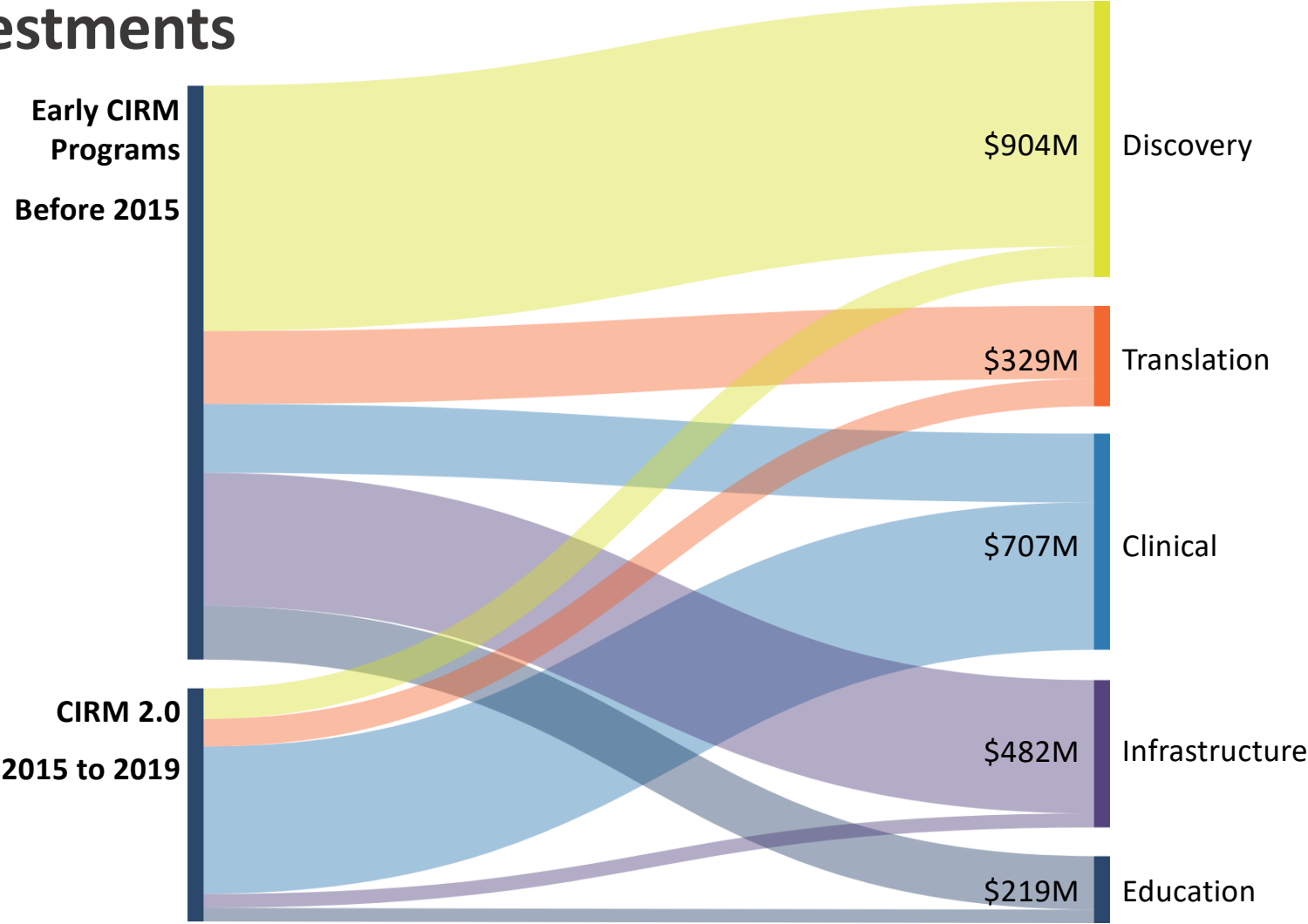
INFRASTRUCTURE



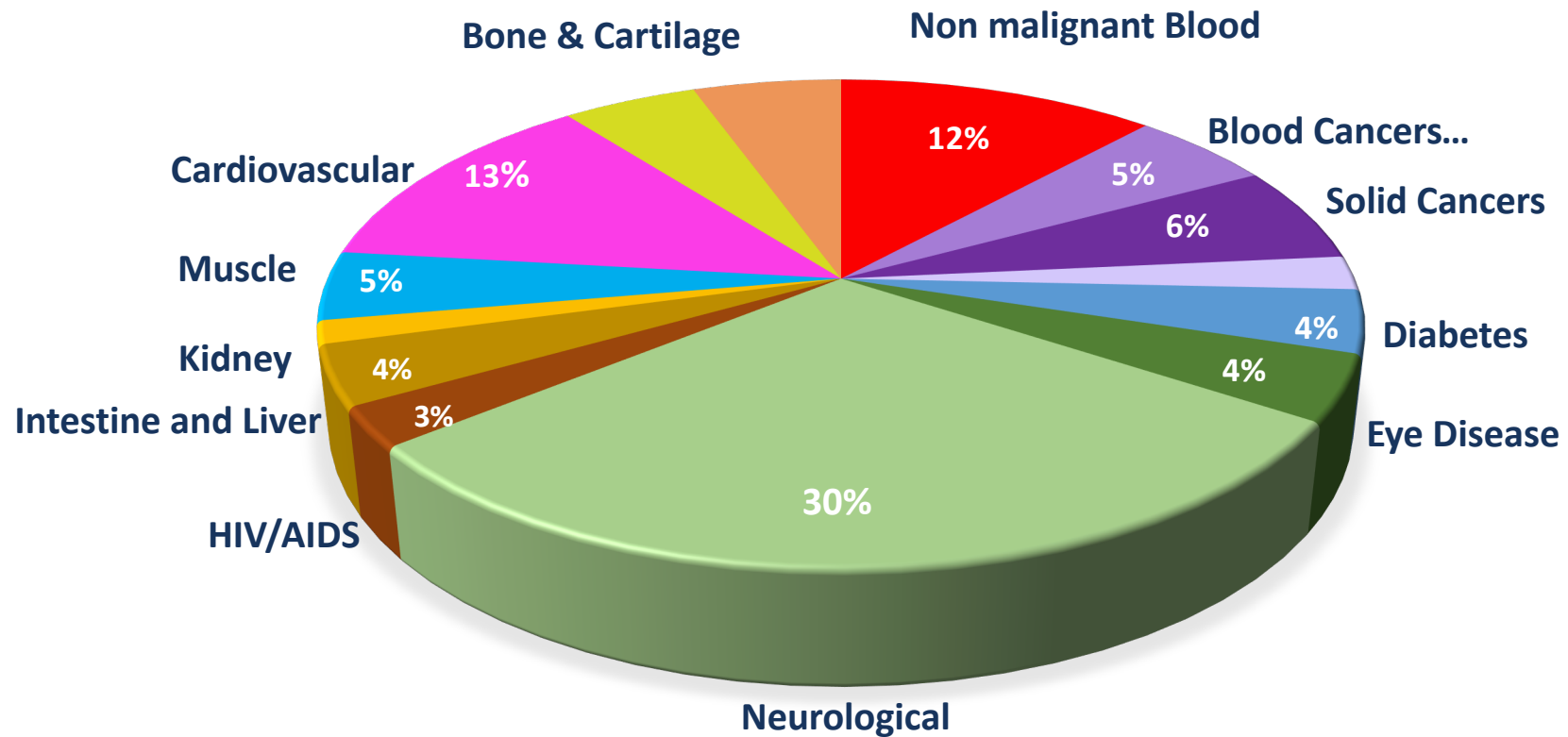
EDUCATION

CIRM Investments

\$2.6B
1000+
Awards

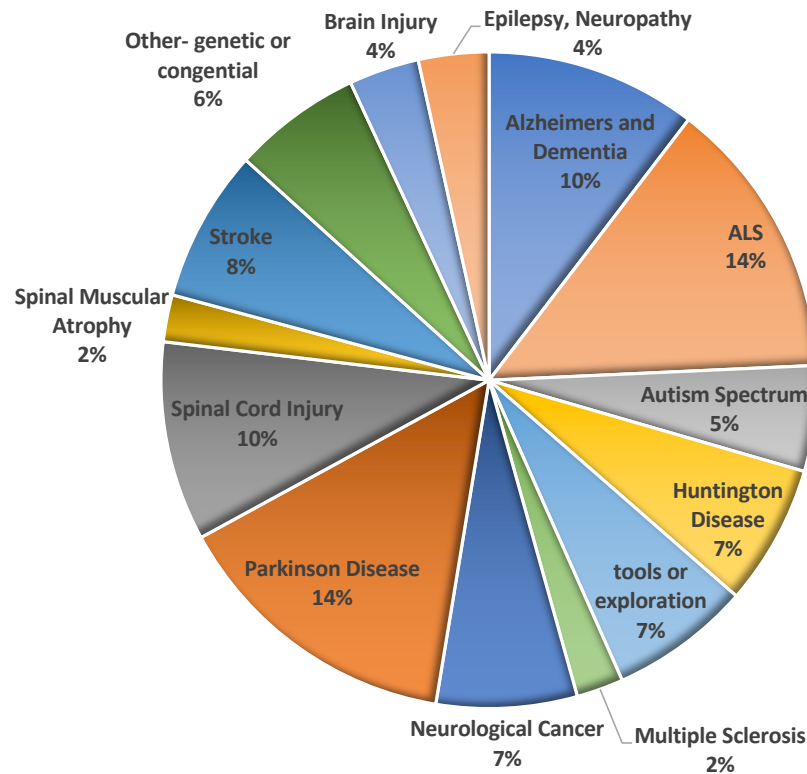


CIRM Research & Development Portfolio



Distribution of CIRM Funding for Neuroscience

CIRM Neuro Funding Across Disease Areas
173 projects, \$422 million



68 Clinical Trials: To Tackle Over 35 Different Unmet Medical Needs



Blinding Eye Disease
Lou Gehrig's Disease
Blood Cancers
Thalassemia
Brain Cancer
Colon Cancer
Heart Disease
HIV/AIDS
Genetic Diseases
Rare Pediatric

Huntington's Disease
Kidney Failure
Lung Cancer
Melanoma
Multiple Myeloma
Bone Disease
Immune Deficiencies
Sickle Cell
Metastatic Cancer
Paralysis
Stroke
Diabetes
COVID-19



Education Pillar : Training Programs 2005-2020

SPARK

HIGH SCHOOL

9 awards
454 trained
\$4M/9 yrs

Bridges

UNDERGRAD/MASTERS

16 awards
1611* trained
\$94M/ 12 yrs

Research Training

PRE/POSTDOCTORAL,
CLINICAL

18 awards
940 trained
\$117M/9 yrs

*Alumni and Presently Completing

Infrastructure Pillar



Buildings and Labs

- Physical Buildings
- Shared Labs

Therapy Development

- Alpha Clinics
- Accelerating Center
- Translating Center

Research

- iPSC Repository
- Genomics Initiative



Stanford Lokey Building



UC Davis GMP facility

Predictable and Recurring Opportunities to Support Therapy Development – Relunched in 2021



DISCOVERY



TRANSLATION



CLINICAL

New Idea



Single Product Candidate



Pre-IND Meeting or Equivalent



Approved Therapy



2/Year

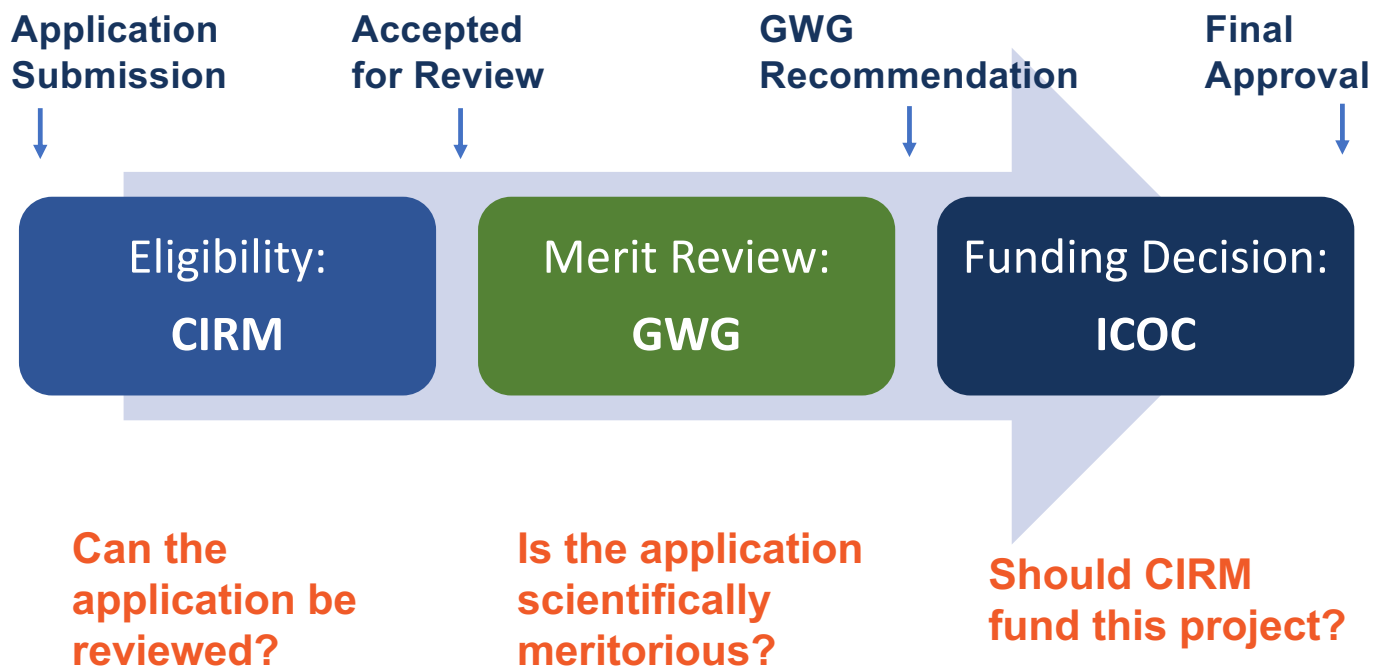


3/Year



12/Year

Application Review Process



Scope of Projects Supported by CIRM

- Stem cell or progenitor cell-based projects
 - Cell therapy development (e.g., hESC, hiPSC, MSC, HSC, and others)
 - Projects directly studying stem/progenitor cells (e.g., mechanisms of differentiation)
 - Projects using stem/progenitor cells as a tool (e.g., disease in a dish models)
 - Directly reprogrammed cells
 - Small molecules and other biologics that act on/depend on stem cells
- “Genetic Research” or Gene Therapy
 - Within scope under Prop 14
 - Is intended to replace, regenerate, or repair the function of aged, diseased, damaged, or defective cells, tissues, and/or organs

Vital Research Opportunity

Prop 14 definition:

“Vital research opportunity” means scientific and medical research and technologies, including, but not limited to, genetics, personalized medicine, and aging as a pathology, and/or any stem cell research not actually funded by the institute under subparagraph (C)...which provides a substantially superior research opportunity, vital to advance medical science as determined by at least a two-thirds vote of a quorum of the members of the Scientific and Medical Research Funding Working Group and recommended as such by that working group to the ICOC, or as determined by the vote of a majority of a quorum of members of the ICOC.

Any research area CIRM would normally not fund that the ICOC deems worthy.

Seeking Feedback from GWG and Other Stakeholders

What should CIRM be thinking about now to prepare for a possible life beyond 2020?

- How can CIRM deliver the greatest impact in the future?
- What opportunities might CIRM seize to accelerate the field?
- What challenges must be addressed?
- What types of CIRM programs should be sustained or expanded?
- What is missing, or needs more support?

Overview of GWG Advice

CIRM is preparing a formal report that will be published on our website and will provide additional detail.

The 5 groups combined proposed a total of 27 recommendations to CIRM.

Some major themes:

- Prioritize funding of work that cannot be funded elsewhere or that is underfunded.
- Explore ways to encourage collaborative, team-based approaches.
- Establish/maintain core services, encourage standardization and implement data sharing.
- Fund all regenerative medicine approaches, and do not limit funding to stem cells.

Overview of GWG Advice

Additional ideas:

- Support infrastructure and technology development for cell and gene therapy manufacturing.
- Enhance training for all levels (high school student to investigators) to address knowledge gaps in regenerative medicine, GMP manufacturing, regulatory affairs, and product development.
- Continue to innovate and improve the peer review process (e.g., revision opportunities, faster response, encourage innovation).
- Establish “moonshot” RFAs to accelerate cures for diseases of greatest impact to the people of California.

CIRM Brainstorming Neurodegeneration 2019

Leveraging Genomics, Stem Cells, Gene Therapy and Novel Clinical Trials for Field-wide Advancement Ideation Workshop

- 2 Day Workshop
- ~50 key opinion leaders representing
 - stem cell R&D
 - Disease foundations
 - Academia
 - Industry,
 - Funding bodies
 - FDA
- **GOAL:** tackle issues specific to ND therapy development using state of the art science to reconfigure discovery and development pipelines and find synergies with groups/consortia

Session Themes

- I: Leveraging Genomics and Big Data
- II: iPSC Models, Creating Standards, Utilizing Banks
- III: Exploring a Neurodegeneration Consortium Model
- IV: Accelerating Drug Development Based on Patient Data
- V: Clinical Trials in Regenerative Medicine – Benefits of a Consortium
- VI: Taking Regenerative Medicine ND Candidates to the Clinic
- Breakout Session: Assessing the Value of a Neurodegeneration Consortium
- Panel Discussion: Would an ND Consortium Accelerate Therapy Development: Considerations for a Path Forward

CIRM Brainstorming Neurodegeneration 2019

Major Thematic Outcomes for Focus

- **Collaborative Science**
 - Partnerships as a mechanism to enhance discovery and development
- **Precision medicine & genomics**
 - current approaches for patient specific development and insights
- **Standardization of practices**
 - enabling data and development comparability from discovery through clinical outcomes
- **Additional high-quality patient derived disease specific iPSC banks**
 - expansion of mechanistically relevant research tools.
- **Novel target discovery and validation**
 - promoting a deeper understanding of disease biology to enable innovative approaches to therapies
- **Expanded biomarker discovery and validation**
 - improving both the predictive and diagnostic biomarkers will lead to improved therapeutic development, testing and ultimately patient care
- **Data harmonization and sharing**
 - sharing common data platforms outcomes of individual investigations to increase in power leading to greater impact
- **Diversity and underrepresented populations**
 - ensuring the above strategies apply to all affected patients beginning with the discovery phase, continuing through clinical development and ultimately with access to treatment