SOMETHING BETTER THAN HOPE

President's Report

Maria T. Millan, M.D.
President & CEO
California Institute for Regenerative Medicine
Report to ICOC- February 2020

CIRM
CALIFORNIA'S STEM CELL AGENCY

CIRM Investments

INFRASTRUCTURE	EDUCATION	DISCOVERY	TRANSLATION	CLINICAL
$480M	$220M	$900M	$365M	$744M

$2.7B and 1000+ Awards

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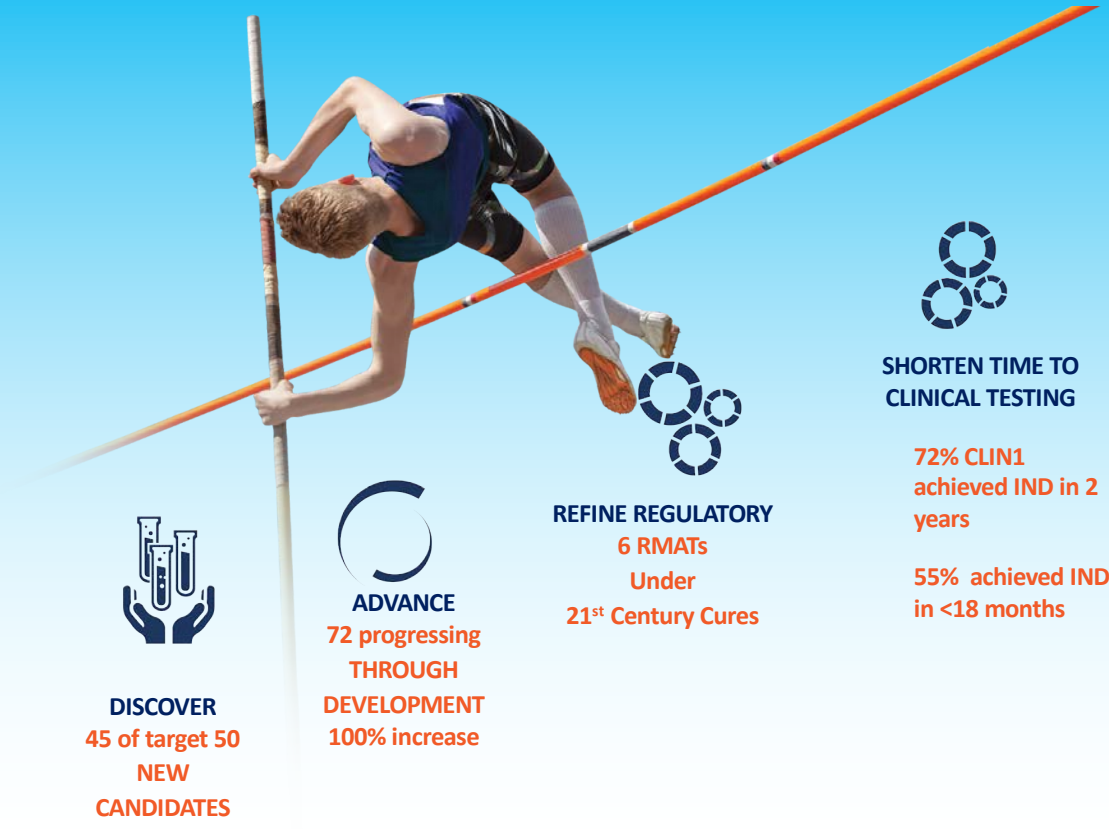
Progress on 5-yr Strategic Plan



DISCOVER
45 of target 50
NEW
CANDIDATES

ADVANCE
72 progressing
THROUGH
DEVELOPMENT
100% increase

REFINE REGULATORY
6 RMATs
Under
21st Century Cures

SHORTEN TIME TO CLINICAL TESTING

72% CLIN1
achieved IND in 2
years

55% achieved IND
in <18 months

EXPAND
43 of target 50
NEW TRIALS
(60 Total Trials
>2000 Enrolled)

INCREASE INDUSTRY PULL
54% partnered
63 events
$3.4 B Industry investment
(brings total leverage
funding to $4.9B)

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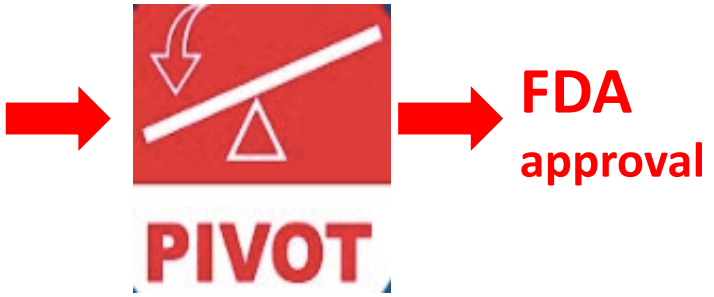
Clinical Update

CIRM Clinical Portfolio: Pivotal Trials

- Pivotal Trials:
  - Humacyte (2 Trials) Phase 3
  - Orchard Phase 2
  - Brainstorm Phase 3
  - Medeor Phase 3
- 'Potential-registration enabling'
  - Forty Seven Inc. Phase 1b
  - Rocket Phase 1/2



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CIRM Clinical Portfolio: Pivotal Trials

ADA Severe Combined Immunodeficiency

Autologous transplant of children with
CD34+ HSC transduced with LV encoding human ADA

Phase 2 Registration Trial
BLA target in 2020
$19M CIRM funding
PI: Spezzi, Orchard Therapeutics
FDA Breakthrough Therapy and Orphan Drug Designation

Clinical Data:
Durable recovery of immune system and 100% event-free survival at 24 months in 20 patients (median age 9 months) who had no other transplant options.

Recent Partnering Highlights: $225.5M IPO (2018), $129.7M Public Offering (2019)

CIRM Clinical Portfolio: Pivotal Trials

PIVOT

Amyotrophic Lateral Sclerosis- Lou Gehrig's Disease

Phase 3, Randomized, Placebo-controlled Multicenter Study to Evaluate Efficacy & Safety of Repeated Administrations of NurOwn® in ALS Patients

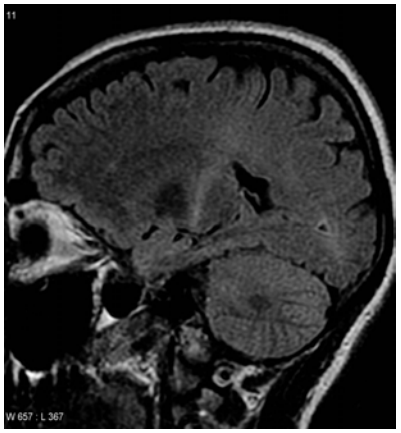
MSC's incubated with a cocktail of cytokines to induce secretion of growth factors and immune modulators and then transplanted into spinal fluid to save neurons targeted by ALS

PI: Ralph Kern, Brainstorm
$15.9M CIRM funding

Fast Track and Orphan Drug FDA designation

Enrollment Completed (n=196 randomized)

Top line data Q4 2020



brainstorm cell therapeutics

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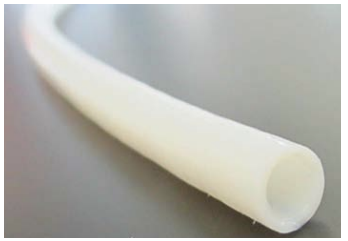
CIRM Clinical Portfolio: Pivotal Trials

Engineered vascular graft for kidney failure

PIVOT

190,000+ Americans on dialysis; 2,200+ US Dialysis Clinics
$24M Total CIRM Funding to develop

- Phase 3 randomized Comparison to PTFE (n=355); enrollment complete
- Phase 3 randomized Comparison to native fistula (n=240); enrolling



HUMACYTE

FRESENIUS MEDICAL CARE

PI: Jeffrey Lawson, Humacyte

FDA RMAT designation (among the first)
Status: Efforts ongoing with FDA regarding BLA submission

Partnership: Fresenius $150M Equity Investment, Global marketing, sales/distribution

CIRM Clinical Portfolio: Pivotal Trials

Inducing Immune Tolerance to Kidney Transplant

PIVOT

Cellular Immunotherapy for Induction of Immune Tolerance in HLA Matched Living Donor Kidney Transplant Recipients

PI: Karen Smith, Medeor

$11.2M CIRM funding

- Phase 3 registrational trial with 30 Haploidentical Patient-Donor Pairs.
- Kidney transplant + conditioning and hematopoietic stem cell transplant
- Enrollment ongoing

Medeor Therapeutics

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CIRM Clinical Portfolio: Pivotal Trials

Gene Therapy for Leukocyte Adhesion Deficiency in Children

PIVOT

PI: Kinnari Patel, Rocket Pharma

$6.6M CIRM funding

Phase 1/2 *registration-enabling* trial. Autologous HSCs transduced with beta 2 integrin gene to treat severe Leukocyte Adhesion Deficiency-1, a rare fatal pediatric disease (n=9)

rocket pharma

'Initial results from 1st patient demonstrate early evidence of safety and potential efficacy'

- Restoration of beta 2 integrin expression
- Visible improvements in multiple disease-related skin lesions after receiving therapy

Recent Partnering Highlights: $85M Public Offering (2019); *Rocket also licensed CIRM funded AAV9 Danon Gene therapy program (2017)*

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CIRM Clinical Portfolio: Pivotal Trials

Novel Immunotherapy Combination Therapy for Leukemia

Phase 1b Trial of Magrolimab in Combination with Azacitidine in Patients with AML/MDS (up to 96)

PI: Mark Chao, Forty Seven, Inc.
$5M CIRM funding

Antibody blocking CD47, a "don't eat me" signal, enables phagocytosis of cancer stem cells by macrophages. Combined with chemotherapy to render the cancer stem cells more susceptible to immune destruction.

- Ongoing Phase 1b single arm trial in myelodysplastic syndrome (MDS) supporting potential registration
- Randomized Phase 3 ENHANCE registration trial to be initiated in 1H 2020
- Fast Track and Orphan Drug designations for MDS

Recent Partnering Highlights:

$113M IPO (2018), $282M Public Offerings (2019)
Ono regional license for Japan and ASEAN territories (2019), Forty Seven-Bluebird Bio collaboration (2019)

Clinical Portfolio: Interim Clinical Data

- Poseida –Multiple Myeloma
- Viacyte –Type 1 Diabetes
- Gottschalk/Sorrentino – X-SCID
- Klassen/JCyte –RP
- Shizuru –Chemo-free conditioning
- Strober-Kidney Transplant
- Kohn –XCGD
- Kipps/Oncternal – Leukemia & lymphoma
- Sangamo- beta thalassemia

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Clinical Portfolio: Interim Clinical Data

Next Generation CAR-T

Phase 1 open label dose escalation multicenter trial to test CAR-T cell stem cell memory cells - engineered via non-viral vector and a “safety switch”

CAR-T to BCMA for relapsed/refractory Multiple Myeloma (n=40)

PI: Matthew A Spear, Poseida Therapeutics

$19.8M CIRM funding

Ph1 trial completed in 2019

FDA RMAT designation

Ph2 *registrational* trial initiated based on positive clinical activity in Ph1 trial

Recent Partnering Highlights: $30.5M Series B (2018), $142M Series C led by Novartis (2019)

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Clinical Portfolio: Interim Clinical Data

ESC-derived cell therapy for Type 1 Diabetes

Phase 1/2 Clinical trial of directly vascularized islet cell replacement therapy for high-risk type 1 diabetes

PI: Howard Foyt, Viacyte

$19.8 M CIRM CLIN2 AWARD

VIACYTE

Insulin and detectable C-peptide in n=8 patients with Type 1 DM after implantation of ESC derived pancreatic cells produce who were C-peptide negative prior to treatment (JP Morgan Showcase Jan 2020)

Recent Partnering Highlights: $80M Series D (2018); CRISPR and WL Gore, Inc. Strategic Partnerships (2017-2018)

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Clinical Portfolio: Interim Clinical Data

Gene Therapy for X-linked SCID

Phase 1/2 trial: Lentiviral Gene Therapy for Infants with X-linked Severe Combined Immunodeficiency using Autologous Bone Marrow Stem Cells and Busulfan Conditioning

PI: Stephen Gottschalk, St. Jude Children's Research Hospital

$11.9M CIRM funding

- LV delivery of normal copy of gene for IL2RG chain
- 24 participants St. Jude/UCSF

FDA RMAT designation

8 patients with 16 month median follow up; normal T cell and NK counts achieved in 4 months post therapy (NEJM 2019)

Recent Partnering Highlights: Licensed by Mustang Bio (2019)

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Clinical Portfolio: Interim Clinical Data

Cell Therapy for Retinitis Pigmentosa

Phase 2b Clinical Study of Safety and Efficacy of Intravitreal Injection of Retinal Progenitor Cells (jCell) for Treatment of Retinitis Pigmentosa (n= >80)

PI: Henry Klassen, UC Irvine /JCyte

$8.29M CIRM funding

- Retinal progenitor cells to rescue light sensing photoreceptors in Retinitis Pigmentosa
- CIRM support of basic discovery, translation, Phase 1 and Phase 2 trials
- Phase 2b Trial completed enrollment

FDA RMAT designation

Initial Phase 1/2a trial showed favorable safety profile and indications of potential benefit and Phase 2b data expected Q2 2020

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Clinical Portfolio: Interim Clinical Data

Non-chemotherapy conditioning for blood stem cell transplant

Phase I trial in SCID: A monoclonal antibody that depletes blood stem cells and enables chemotherapy free transplants (N=24)
PI: Judith Shizuru, Stanford
$19M DR2A CIRM funding
$3.43 CLIN2

Stanford MEDICINE

Interim results:

After 6 months, 4 of 6 patients reached the predefined endpoint of chimerism & blood stem cell engraftment (ASH Dec 2019)

This is the first demonstration of HSC engraftment without the use of radiation or chemotherapy conditioning

Recent Partnering Highlights: Jasper Therapeutics licensed technology with $50M Series A led by Roche Venture Fund, Abingworth and Qiming (2019-20)

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Clinical Portfolio: Interim Clinical Data

Immune Tolerance to Kidney Transplantation

Combined Kidney and Hematopoietic Progenitor Cell Transplants from Haplotype Matched Living Donors

PI: Samuel Strober, Stanford

Stanford MEDICINE

$6.65M CIRM funding

- Donor HSC and T-cell transplantation to induce mixed chimerism
- 24 of 29 patients with persistent mixed chimerism for ≥6 mo

Persistent mixed chimerism established in fully HLA- or haplotype-matched patients allowed for complete or partial withdrawal of immunosuppressive drugs without rejection (Sci. Transl. Med. 12, 29 Jan 2020)

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Clinical Portfolio: Interim Clinical Data

Cell-Gene Therapy for X-linked Chronic Granulomatous Disease

A Phase 1/2, Non-Randomized, Multicenter, Open-Label Study (n=10)

PI: Don Kohn, UCLA

$7M CIRM funding for the Phase 1/2 Clinical Trial

UCLA

Orchard therapeutics

Clinical Results:

- 6 patients with sustained levels of functioning neutrophils at 1 yr.
- No new CGD-related infections w/ no antibiotic prophylaxis (Nature Medicine Jan 2020)

Licensed by Orchard- $225.5M IPO (2018), $129.7M Public Offering (2019)

Granted Orphan Drug Designation

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Clinical Portfolio: Interim Clinical Data

Targeting cancer stem cells in Leukemia and Lymphoma

Phase 1/2 Clinical Study of Cirmtuzumab and ibrutinib in Patients with chronic lymphocytic leukemia (CLL) and mantle cell lymphoma (MCL) (n= >136)
PI: Thomas Kipps, UC San Diego/Oncternal
$18.29 M CIRM funding

Phase 2 enrolling
Interim results from Phase 1:

ONCTERNAL therapeutics™

MCL cohort:

- Best Objective Response Rate of 66.7% (6 of 9 evaluable)
- Complete response rate of 33.3% (3 of 9 evaluable)
- All 3 CRs in heavily pretreated patients

CLL Cohort

- Best Objective Response Rate of 85%
- 1 confirmed complete response and 3 clinical complete responses
- No progressive disease observed at median follow-up of 7.4 months for Progression Free Survival of 100% (2019 ASH Meeting, updated Jan 2020)

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Clinical Portfolio: Interim Clinical Data

Gene-edited cell therapy for beta thalassemia

Phase 1/2 Study to Assess the Safety, Tolerability, and Efficacy of gene modified autologous blood stem cells to increase fetal hemoglobin as treatment for transfusion-dependent β-Thalassemia (n=6)

PI: Weston Miller, Sangamo BioSciences, Inc.

$8M CIRM funding

Sangamo THERAPEUTICS

Preliminary results from 3 patients showed prompt hematopoietic reconstitution, on target gene editing in circulating cells and evidence of HgF synthesis, indicating successful gene editing. Longer term follow-up ongoing to assess clinical significance.

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Clinical Portfolio: New INDs 2019

- ✓ Fate therapeutics: Engineered iPSC derived NK Cells for Solid Tumors
- ✓ Deng/UCLA: Autologous limbal stem cells for Corneal damage
- ✓ Poseida: CAR-T targeting PMSA-1 for castrate resistant metastatic Prostate Cancer
- ✓ Zaia/City of Hope: Chemotoxicity resistant modified HSCs for Glioblastoma treatment
- ✓ Steinberg/Stanford: hESC derived neural stem cells for chronic subcortical ischemic stroke
- ✓ Wu/Stanford: hESC derived cardiomyocytes for end stage Heart Failure

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CIRM Clinical Portfolio: Sickle Cell Update

- CIRM and NHLBI MOU to jointly fund industry and academic cell and gene programs for the Cure Sickle Cell Initiative
- CIRM Alpha Clinics part of the ASH Sickle Cell trial network & data hub
- First award (Walters) funded in this MOU 50/50 - on time; no delay in contracting
- At least Six Applicants preparing for 2020 CLIN Submission (3 industry; 3 academic)

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CIRM Clinical Portfolio: Sickle Cell Disease

Kohn (UCLA)	**Phase 1 LV gene addition (anti-sickling globin protein)** **CIRM funding $13M; n=6 patients**
Rosenthal (City of Hope)	**Phase 1 mild conditioning for haplo-match BMT** **CIRM funding $5.7M; n=6 patients**
Porteus (Stanford)	**IND-enabling CRISPR/Cas 9 gene editing (Val to Glu)**
Walters (CHORI) 	**IND-enabling CRISPR/Cas9 gene editing (Val to Glu)**

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Right now.

Paul Webb PhD
CAP Program Manager
California Institute for Regenerative Medicine
2020

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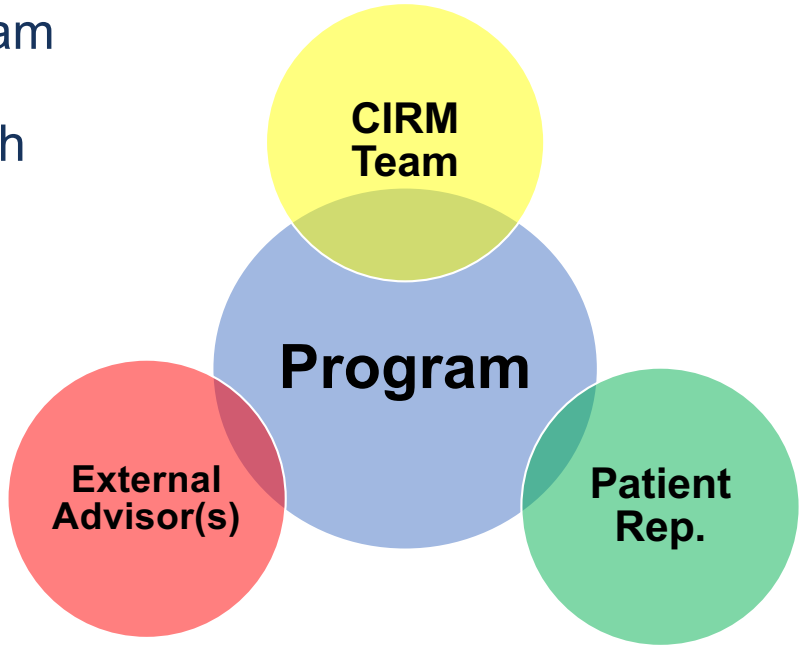
Clinical Advisory Panels (CAPs)

- **Purpose** of a **CAP** is to provide guidance and advice to the project team
- A **CAP** is assembled by CIRM for each Clinical Stage Award
- A **CAP** is composed of:
  - CIRM team
  - External advisors
  - Patient reps
- Multiple **CAP Meetings** occur over lifetime of a program award



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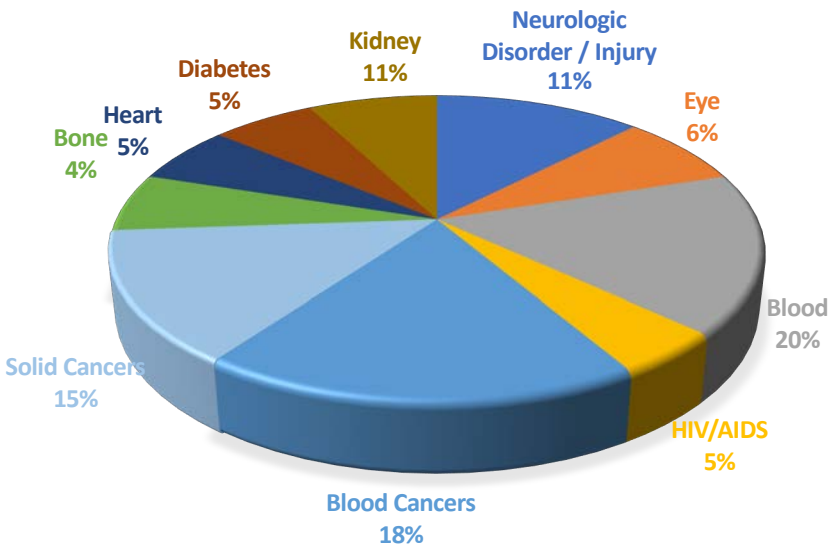
CAPs - A Key Tool in Active Management of CIRM Clinical Portfolio

- **CLIN1 Awards: 22 Panels**
  - Submit an IND
  - Duration 18-24 months
- **CLIN2 Awards: 56 Panels**
  - Complete Clinical trial
  - Duration up to 4 years

CIRM Clinical Trials Profile



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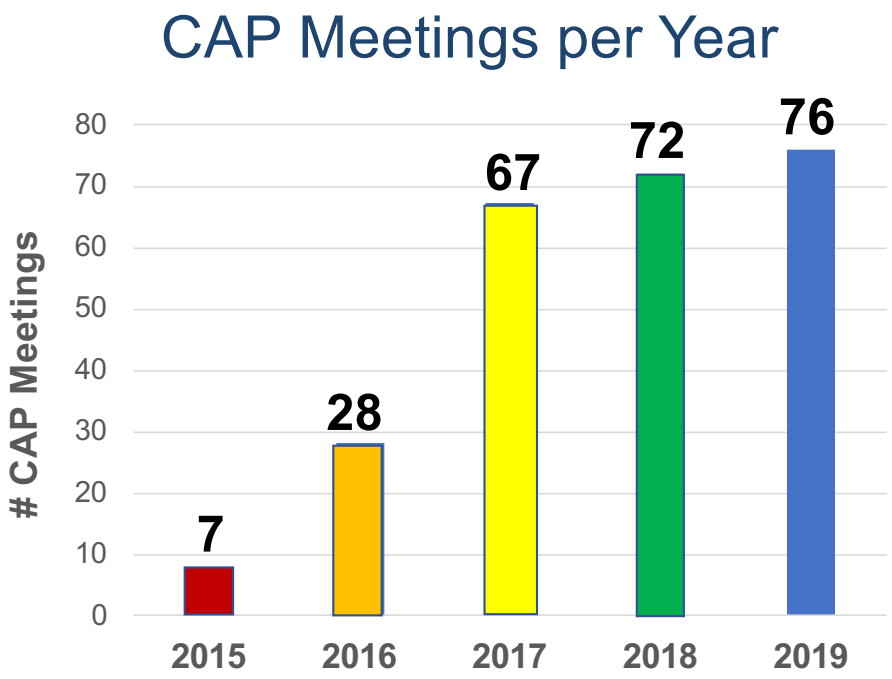
CAP Meetings-Statistics



- **250** CAP meetings since inception
- **78** External advisors
- **57** Patient reps

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Did CAPs Make a Difference?

Impact Definition – A CAP
Feedback that helped the team

Types of Impact:

- Resolving specific challenges
- Optimizing project execution
- Discovering critical information

Outcomes

- Manufacturing challenges overcome
- Clinical trial design optimized
- Enrollment enhanced
- Regulatory advice provided
- Partnering facilitated
- Development Path delineated

To date, CAPs influenced 74% of CLIN awards

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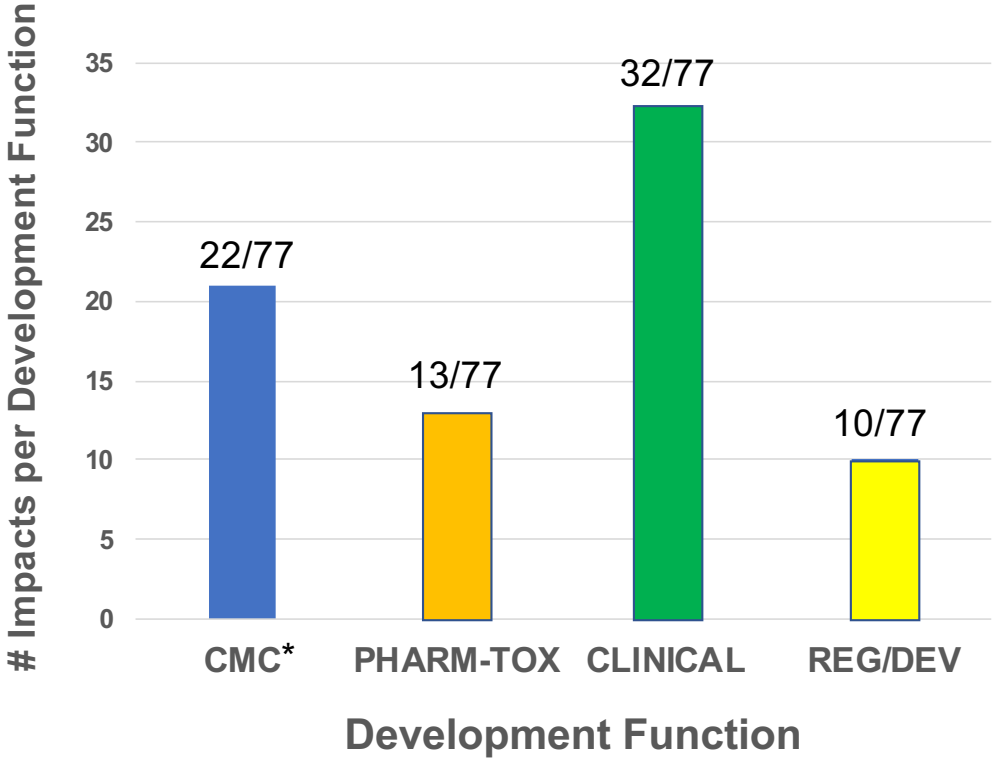
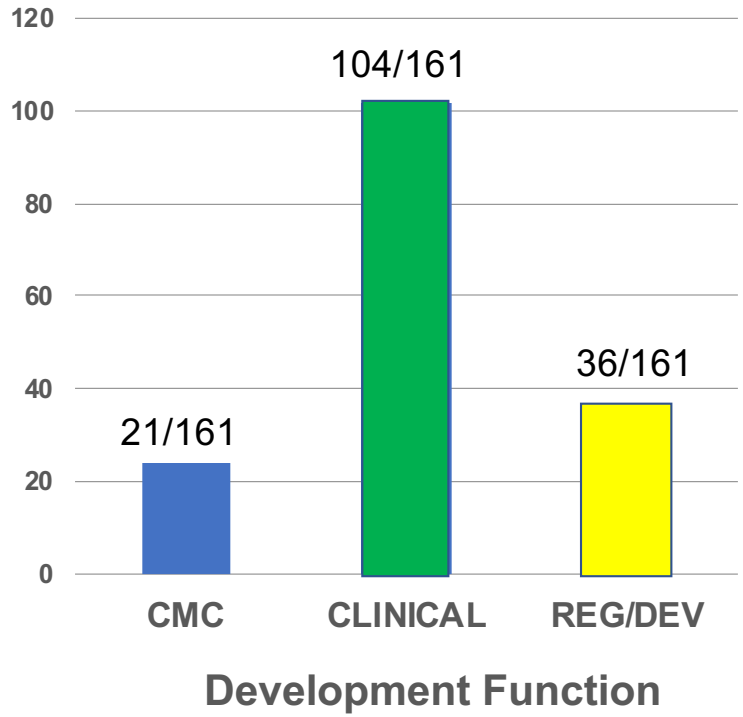
CAPs Influenced Key Development Functions

CLIN1 – 77 Impacts



CLIN2 – 161 Impacts



*CMC=Manufacturing

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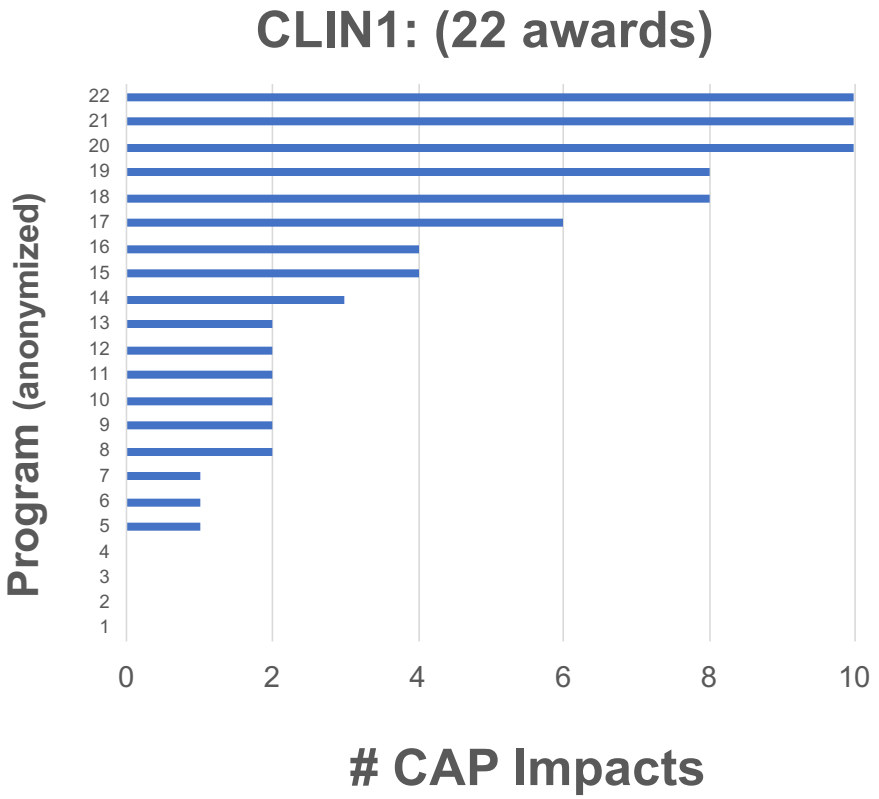
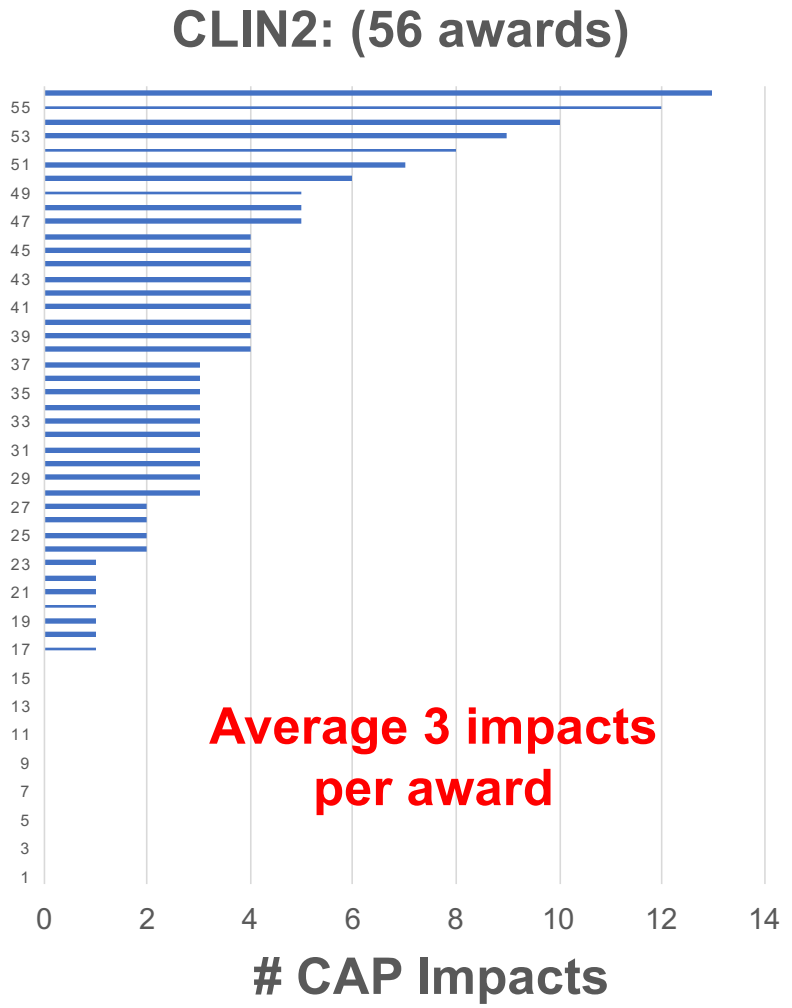
CAP Impacts per Award





Average 3 impacts per award

Variability Driven by Program Needs and Launch Date

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Right now.

Kent Fitzgerald PhD
Director of Discovery & Translation
California Institute for Regenerative Medicine

February 6, 2020

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Translational Program Overview

- **Expected Outcome**: At the conclusion of a TRAN award is the start of a well-prepared pre-IND meeting or equivalent meeting with the FDA, resulting in correspondence from the FDA confirming agreement with the IND-enabling preclinical plan.
- **Timeline**: 30 MONTHS (MAX)
- **Budget**: $4M cell therapy, $2M small molecule
- **Readiness**: single eligible human clinically compatible candidate with:
  - Reproducible disease- modifying activity relevant to the target clinical indication(s)
  - Consented for research and commercial use
  - Meeting donor eligibility requirements (per FDA HCT/P guidance)

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TRAN Program Overview

- **Allowable activities: <u>critical path for meeting expected outcome</u>**
  - cGMP activities
    - GMP-compatible process scale-up/development
    - Preparation of cGMP-compliant Master and Working cell banks
    - Tech transfer to manufacturing
  - Assay development and qualification (e.g. in-process and release assays; stability, activity, tumorigenicity and immunogenicity assays)
  - Biomarker development
  - Conduct of non-clinical studies including pharmacodynamic, pharmacokinetic (cell biodistribution/fate), immunogenicity, pilot safety and mechanism of action (MOA) studies
  - Studies to select dose, determine regimen and route of administration
  - Selection of indication, development of a clinical plan, including a clinical protocol synopsis and draft protocol, for a phase 1 trial

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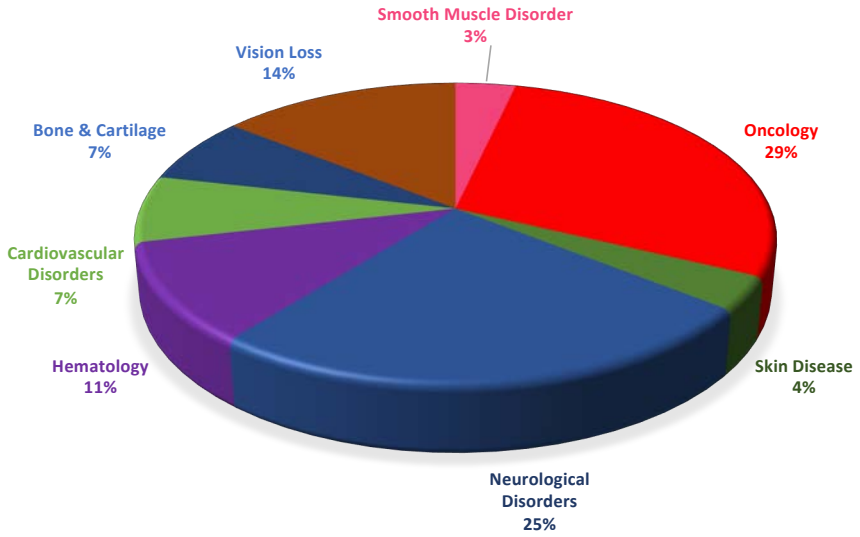
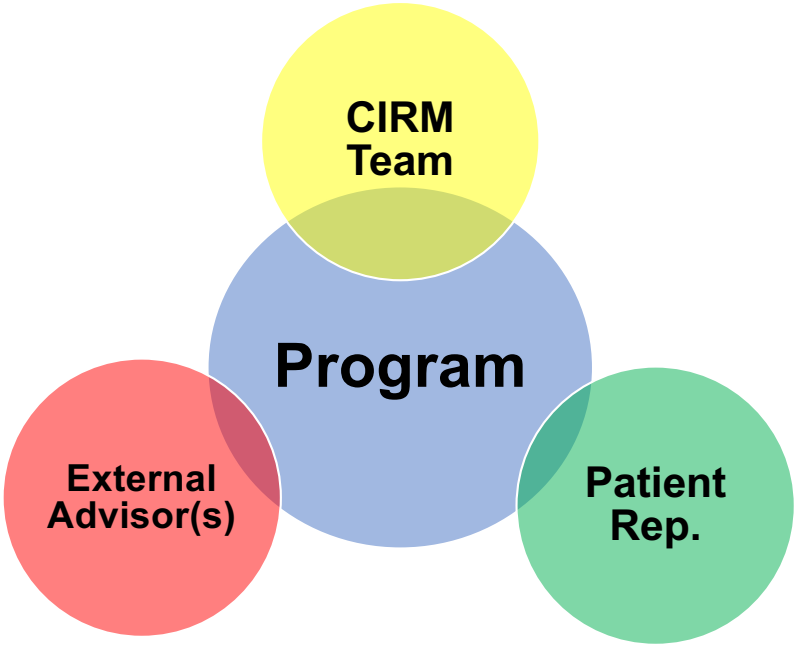
TRAN Program Stats



- Started in June 2016
- Number of Programs = 31
  - 83 Operational Milestones associated with active projects
- 100% of concluded TRAN projects have held successful pre-submission meetings (n=5 to date)
- To date 2 TRAN have progressed to CLIN1

Translational Advisory Panels (TAPs)
Composition & Definitions



- A **TAP** is the panel (CIRM staff + advisors) assembled to work with a specific TRAN project
- Multiple **TAP meetings** occur over lifetime of a TRAN project
- A **TAP Impact** is a TAP feedback/recommendation that results in a positive impact on achievement of the project objective
- The TAP can have multiple impacts on a TRAN project over its lifetime

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TAP Meetings Statistics and Outcomes

Statistics

- Started in July 2018
- **7** TRAN projects with a TAP since program inception
- **9** Total TAP Meetings conducted
- **16** Advisor Specialists
- **6** Patient Representatives
- **64** Impacts Documented

Outcomes

- cGMP Process Development Challenges Resolved
- Pre-clinical Study Design Optimized
- Targeted Regulatory Advice
- Regulatory Submission Review
- Development Path Refined
- Partnering Facilitated

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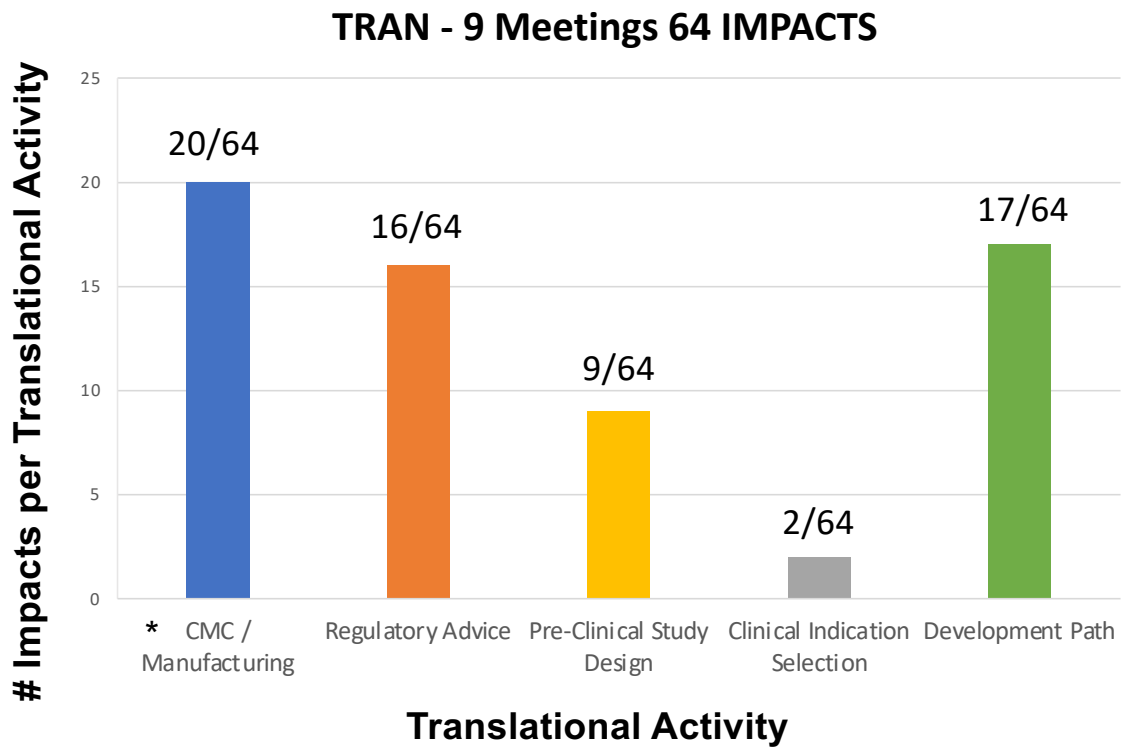
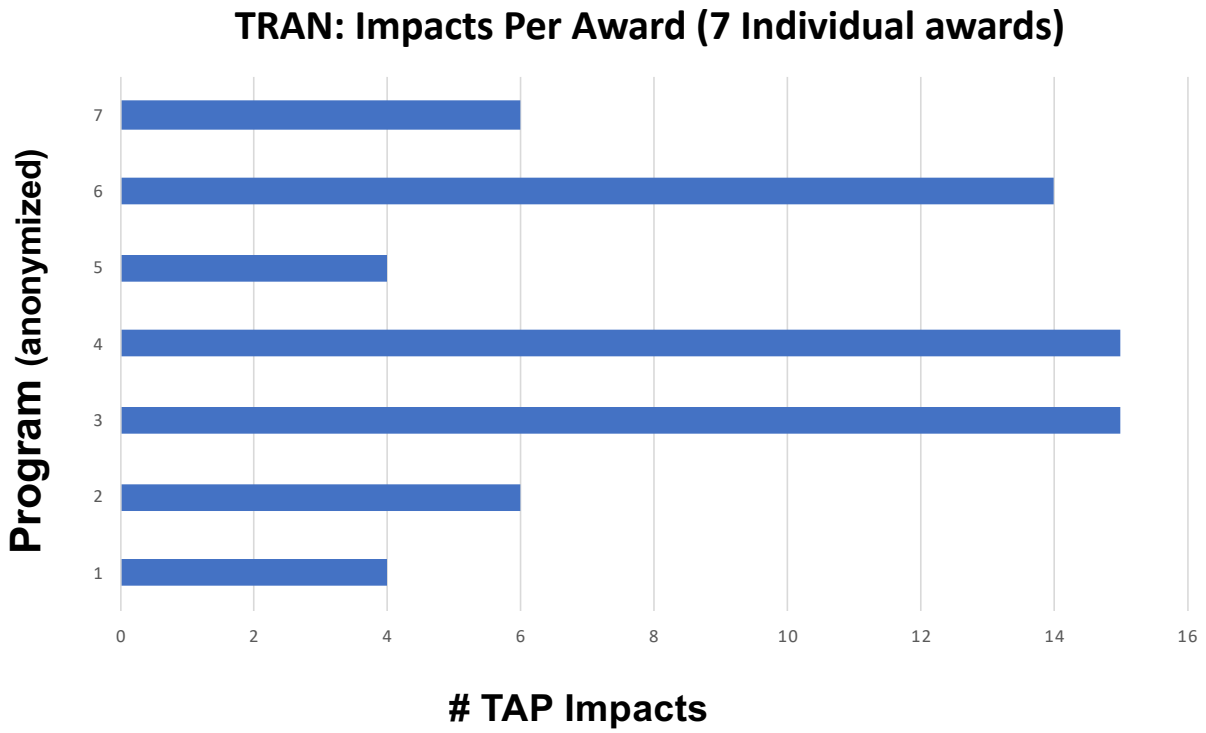
TAPs Influenced All Key Translational Activities



*CMC=Process Development

Display of TAP Impacts Per Award



Variability is Driven by Differences in Program Needs and Maturity Upon TAP Implementation

Kelly A. Shepard, Ph.D.
Associate Director,
Discovery and Translation

February 6, 2020

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2004

$3B Bond funding -Proposition 71

1000+

Projects Funded

60

Clinical Trials

2000+

Patients Enrolled

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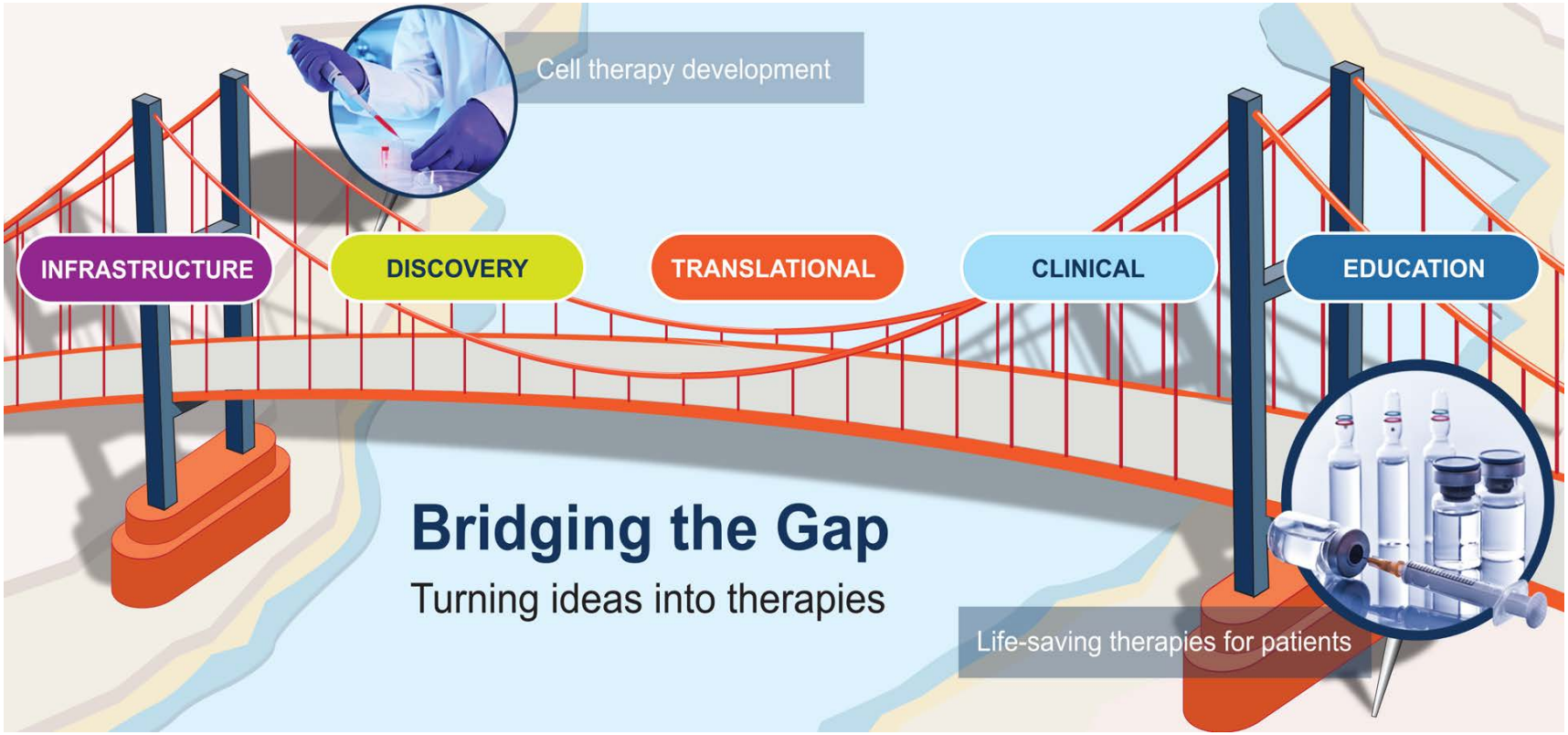
CIRM Investments: 5 Pillars



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Creating a diverse, well-trained workforce of scientists and technicians to help realize the full potential of stem cells to treat patients with unmet medical needs

The Bridges Program

UNDERGRAD/MASTERS

The SPARK Program

HIGH SCHOOL

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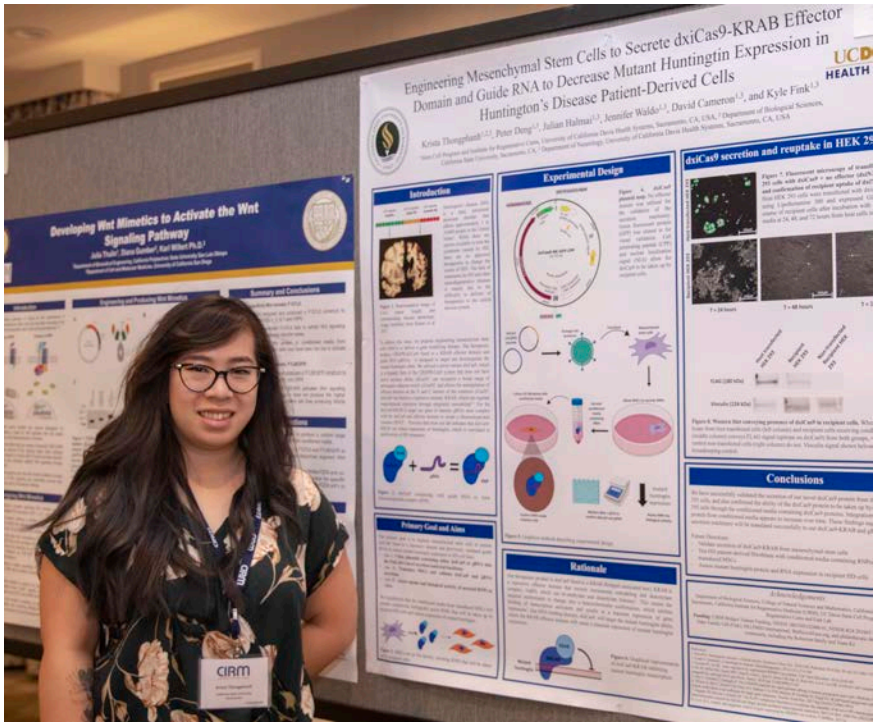
Bridges Program: Undergraduate and Masters Level Training

Objective: To prepare California's undergraduate and master's graduate students for highly productive careers in stem cell research and therapy development

Structure: Programs are integrated into bachelors, masters or certificate programs based at “home” institutions around the state of CA



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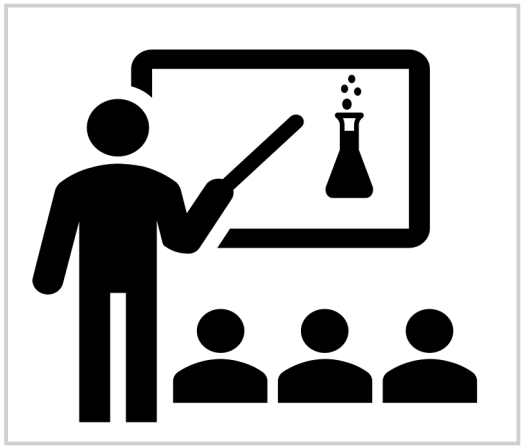
Bridges Program: 16 Home Universities Across California

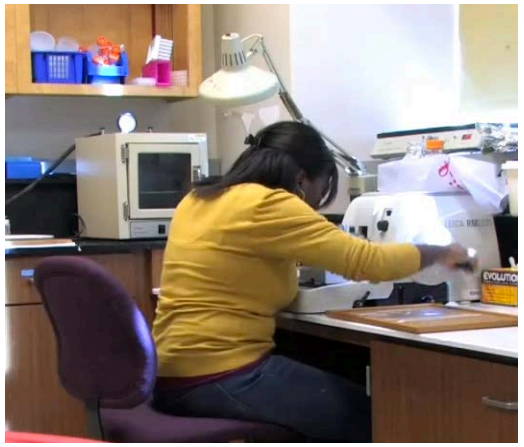
- 14 currently active programs

Bridges: Core Program Features



Coursework and Laboratory Techniques

Patient Engagement and Community Outreach



Paid Research Internships at Host Institutions

CIRM Annual Bridges Conference

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Bridges Alumni: Outcomes and Impact

- 1373 alumni in first 10 years
- 48% first generation college students
- > 60% employed in R&D positions
  - 53% academic labs
  - 47% biotech/pharma
- About 35% in PhD, professional, or other graduate programs, including medical school
- Contributed to 261 publications in scientific journals while in program

10th Annual Bridges Conference
July 2019, San Mateo, CA

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Bridges Program: Success Stories

Michael Silva, MS

Solano Community College
UCSB
CSU Channel Islands
City of Hope Intern
Genentech
Professor of Biotechnology at Solano Community College

Vahid Hamzeinejad, MS

Left UC Berkeley to help family
Community College
Transferred to Cal Poly
Viacyte Intern
Manufacturing Engineer, Viacyte

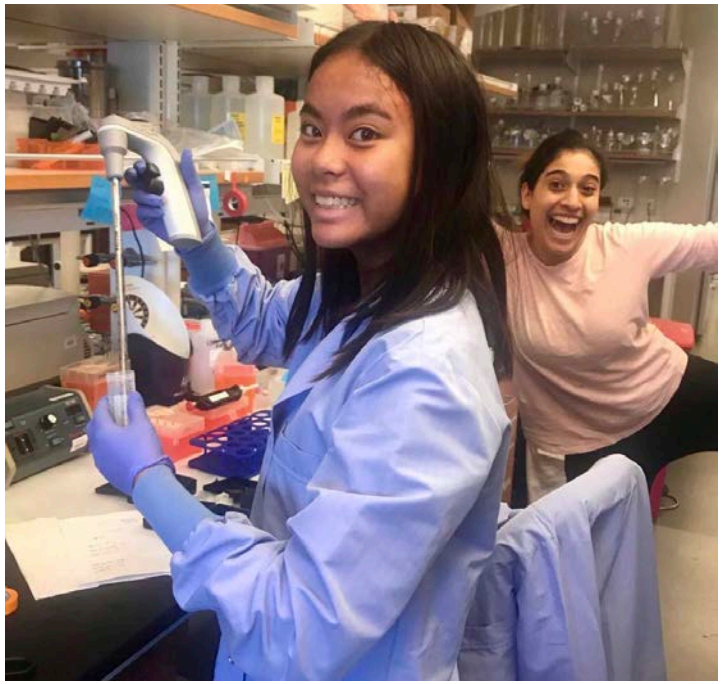
Laughing Bear Torrez, PhD

CSU San Bernardino
UC Riverside Intern
Stanford Stem Cell PhD, 2019
Scientist at Bolt Biotherapeutics

SPARK Program: High School Level Training

Objective: To provide high school students with hands-on training in stem cell research through summer internships and to inspire their interest in regenerative medicine

Structure: SPARK programs supplement and integrate within existing summer programs sponsored by eligible California institutions



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SPARK/Creativity Awards: 10 Programs Across California

UCSF

GLADSTONE INSTITUTES

C·H·O·R·I
Children's Hospital Oakland Research Institute

UCDAVIS
UNIVERSITY OF CALIFORNIA

Stanford University

UCSB

Caltech

City of Hope

CEDARS-SINAI

The Scripps Research Institute

- 7 currently active programs

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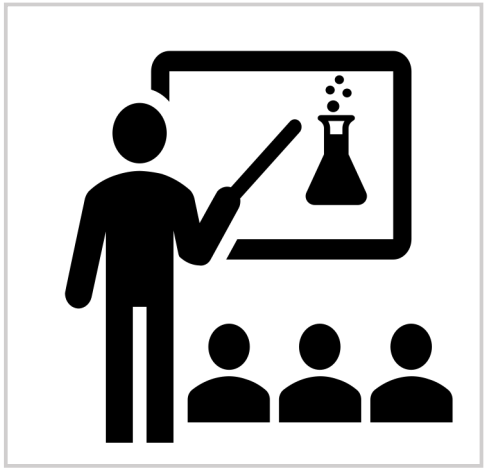
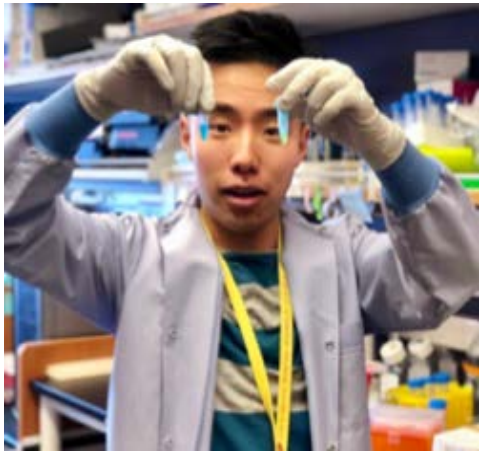
SPARK: Core Program Features

Preparatory Courses and Workshops



Patient Engagement, Community Outreach and Social Media

Summer Research Internship



Annual SPARK Poster Day

SPARK Program: Outcomes and Impact

- 482 students have completed internships since 2012
- Many trainees are still in high school, but of 76 recent of alumni who reported college attendance:
  - 50% are attending a UC
  - 18% attending another CA school (Stanford, Caltech, CSU)
  - 32% attend schools outside CA (Yale, Columbia, Harvard, Johns Hopkins, Duke, etc.)
  - Most pursuing biology or other STEM related fields

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Bridges and SPARK Programs

EDUCATION

More information:
https://www.cirm.ca.gov/education

Thank you.

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Planning for a "Beyond 2020" Strategic Plan
Proposed Themes for Board Discussion

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Strategic Planning Process: Broad Outline

Identify critical gaps and opportunities from ICOC, outreach, workshops, and public input.

Develop approach via new concept proposals or refinements to current programs to address these gaps and opportunities.

Define action plan and create measurable goals against this plan.

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Proposed Strategic Themes

Mission:

Accelerate stem cell and regenerative medicine treatments to patients with unmet medical needs

- Advance world class science and development of therapies
- Build Pathways to Commercialization
- Increase Access to Patients
- Maximize Funding Through Operational Excellence

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Theme 1: Advance World Class Science

Refinements to 5 Funding Pillars:

- Discovery: fundamental and necessary component
- Translation: Build upon CIRM "value proposition"
- Clinical programs: "next generation" programs
- Education: Workforce Development
- Infrastructure: Accelerate maturation of field and delivery to patients

Identify areas that do not fit into the 5 Pillars.

Identify prior program and infrastructure investments that can be leveraged for future research programs.

Consider consortia or "moon shot" approaches.

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Theme 2: Build Pathways to Commercialization

Address manufacturing needs for regenerative medicine products.

Promote best in class investment & commercialization partnerships.

Partnership with grantee institutions to optimize IP & commercialization potential of CIRM funded programs.

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Theme 3: Increase Access to Patients

Increase patient awareness and access to FDA regulated clinical trials and newly approved regenerative medicine therapies

Evidence generation to support regenerative medicine adoption and coverage

Data and knowledge platforms to support access

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Theme 4: Maximize Funding Through Operational Excellence

In addition to reviving DISC/TRAN/CLIN RFA's should there be funding beyond 2020…

Propose PAs/RFAs that support Strategic Themes

- Structure for a Consortium Approach
- Operationalize funding partnerships (as was done with NHLBI)
- Support new PA and specialty review needs

Refinements to Review Process and Award Management

IT Enhancements

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RELENTLESS

DRIVE

DELIVERING

CURES

ADVOCATING

CHANGE

UNWAVERING

RESOLVE