



**Accelerating Stem Cell Treatments to
Patients with Unmet Medical Needs**

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President and CEO
The California Institute for Regenerative Medicine

CIRM's Mission

Beyond
CIRM_{2.0}
CALIFORNIA / STEM CELL AGENCY
now it's personal

Accelerate stem cell treatments to patients with unmet medical needs

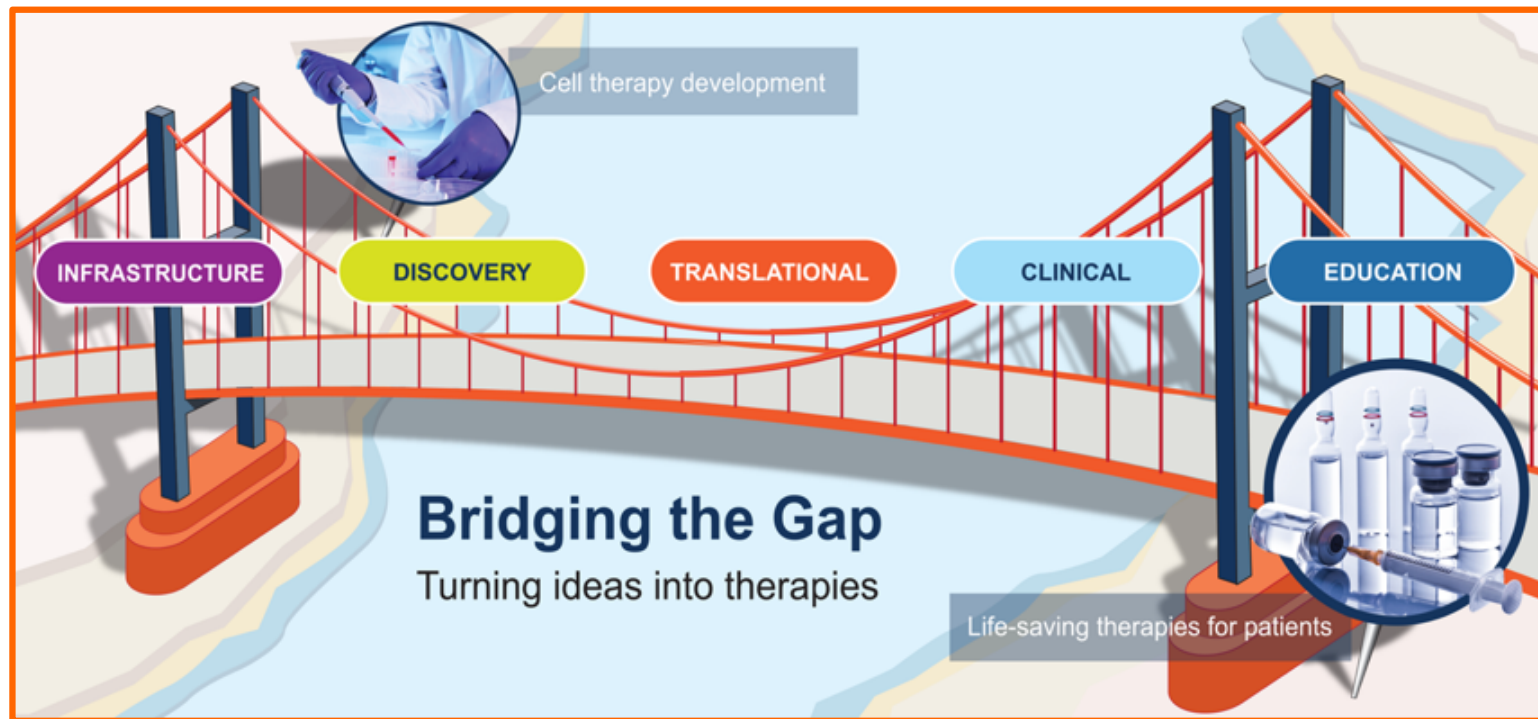
CIRM_{2.0}
CALIFORNIA / STEM CELL AGENCY

All In All Out | Every Moment Counts

CIRM Value Proposition



5 Pillars of investment accelerate science to treatments

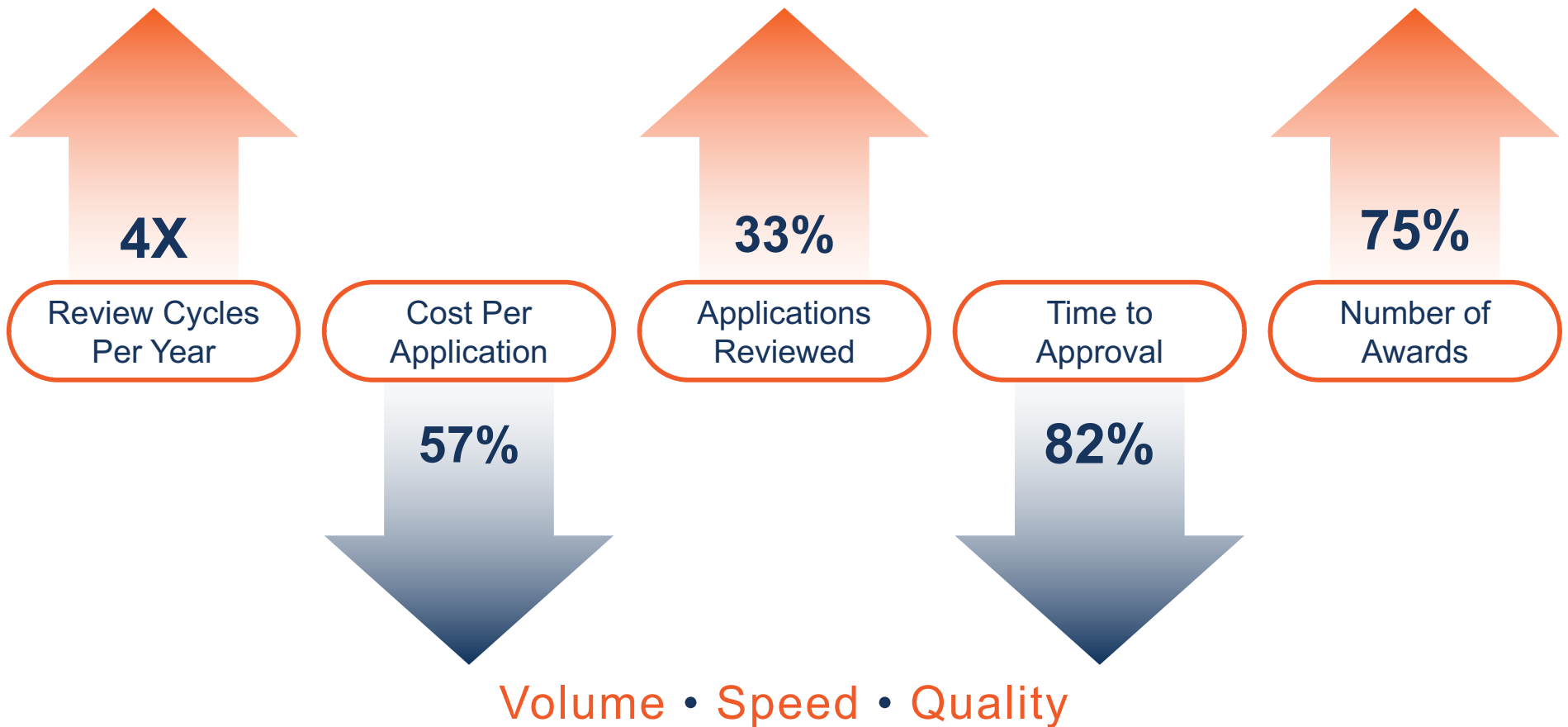


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CIRM Value Proposition



Acceleration through CIRM 2.0 Operational Excellence



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Objective Measures of Success

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VISION | 5-YEAR GOALS |

DISCOVER

**50
NEW**

therapeutic or device
candidates

ADVANCE

**50%
MORE**

Projects advancing
To next stage

REFINE

**ENACT
BETTER**

more efficient
Regulatory
paradigm

ACCELERATE

**50%
LESS**

Time to move from
Discovery into
clinical trials

VALIDATE

50

50 new clinical
trials covering 20
unique diseases or
conditions

PARTNER

**+50%
PARTNERED**

clinical stage
Programs with
commercial partners

*Exponentially advance CIRM's Mission
by defining the path forward, and leading the way*

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Update on “Risks” vs Performance



Risks

- Enough meritorious programs for 50 new trials in 5 yrs ?
- Interest from qualified applicants ?
- Limited CIRM Prop 71 funding on recruitment/retention ?
- Insufficient investor interest in cell therapy ?
- Regulatory Hurdles ?

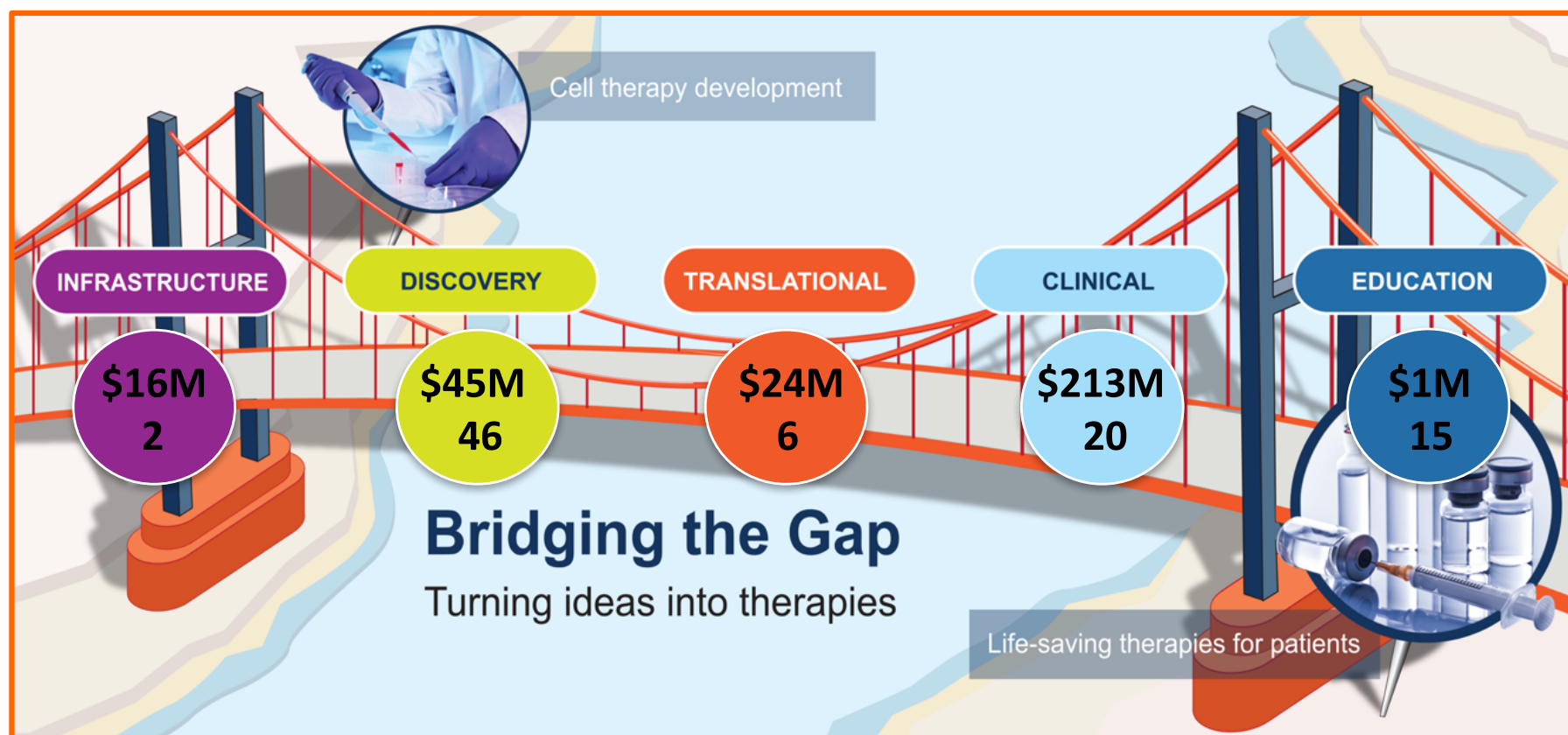
Performance

- In 2 years, 26 new trials!
- Record #'s high quality applications!
- Retaining & recruiting top talent!
- Investment/deals: 3 in 2016 and 6 in 2017!
- 21st Century Cures Act- Dec. 2016
CIRM has 3 of the 11 expedited FDA RMAT designations thus far !

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The CIRM Spectrum

2017 Estimated Investment and # of Awards



Leveraging CIRM Investment



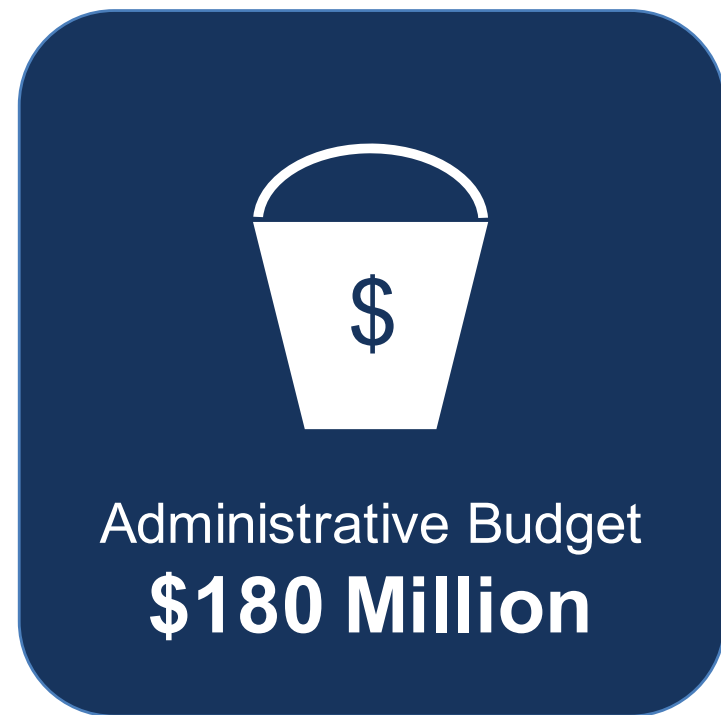
CIRM's early investment attracts follow-on funding

Co-funding by Applicant	Additional Funds due CIRM Award	Resulting Partnership Funding
\$911M *\$50M from non-CA	\$395M	>\$397M

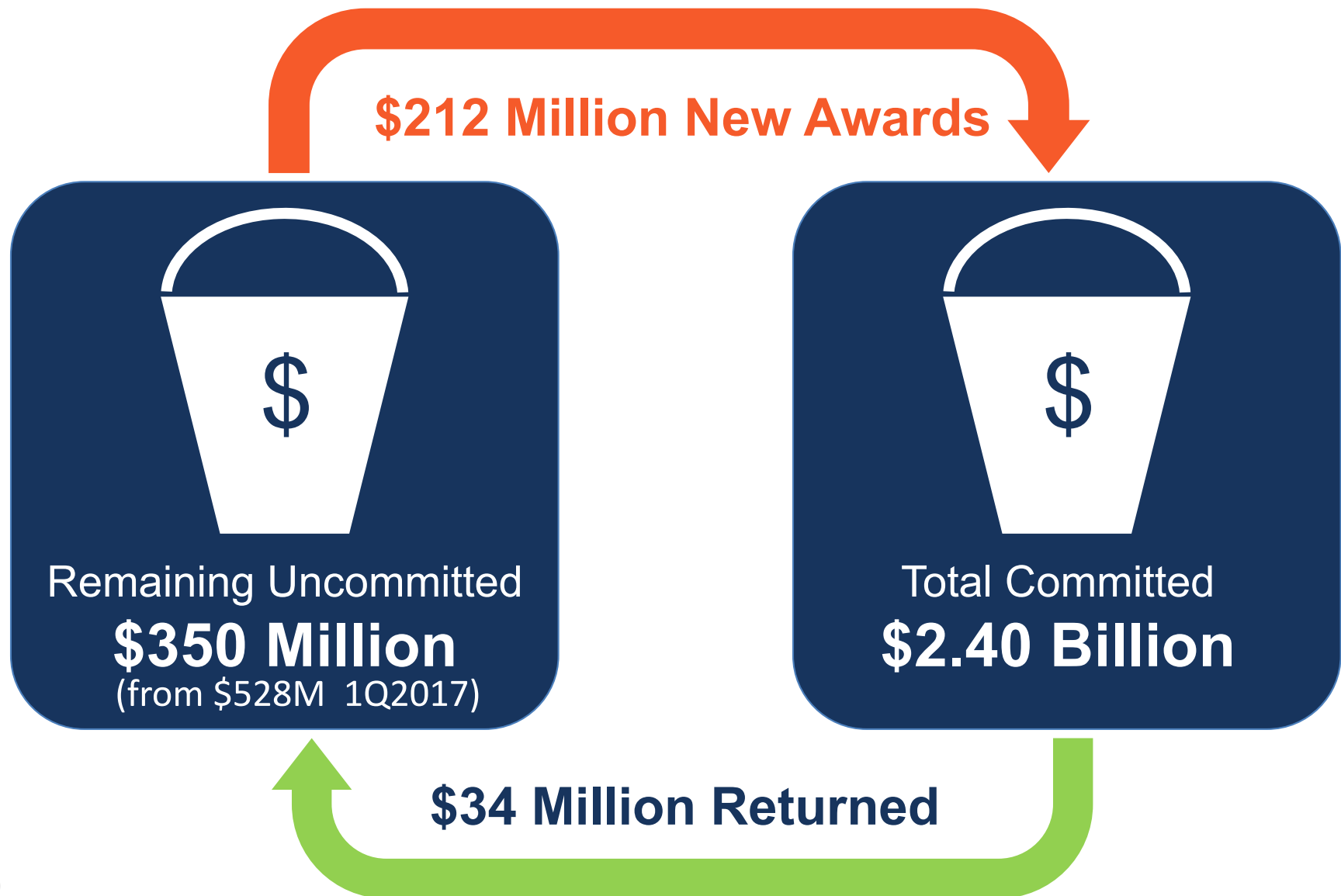
* Approximate

CIRM Budget from 2 Buckets

Award + Administrative

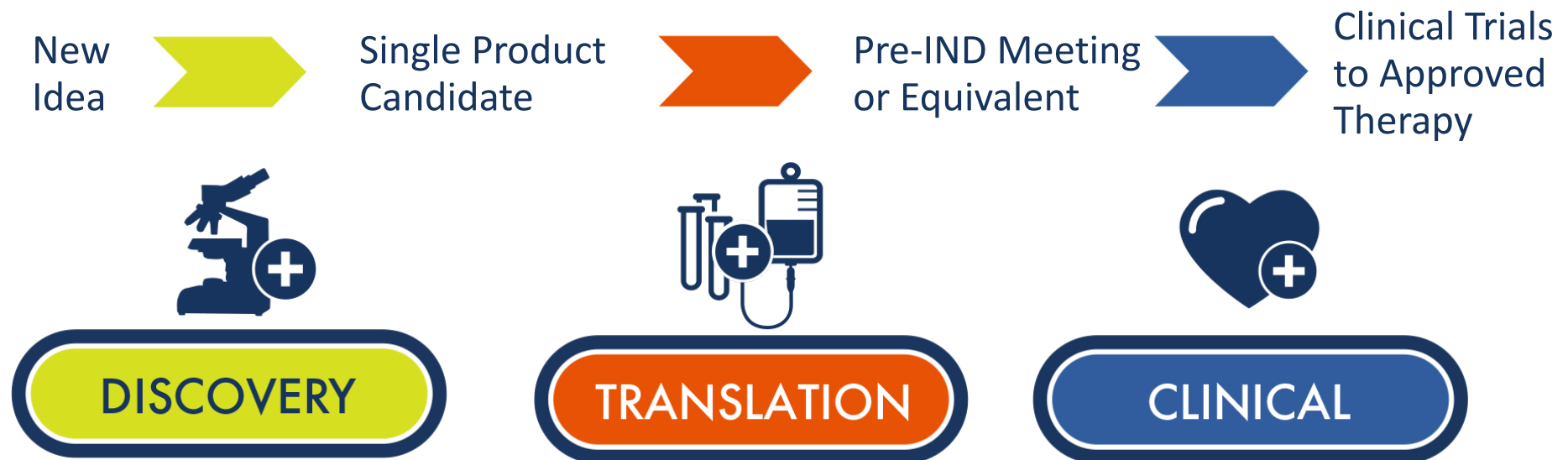


Research Award Bucket through Q3 2017



CIRM Research & Development Program Offerings: 2017

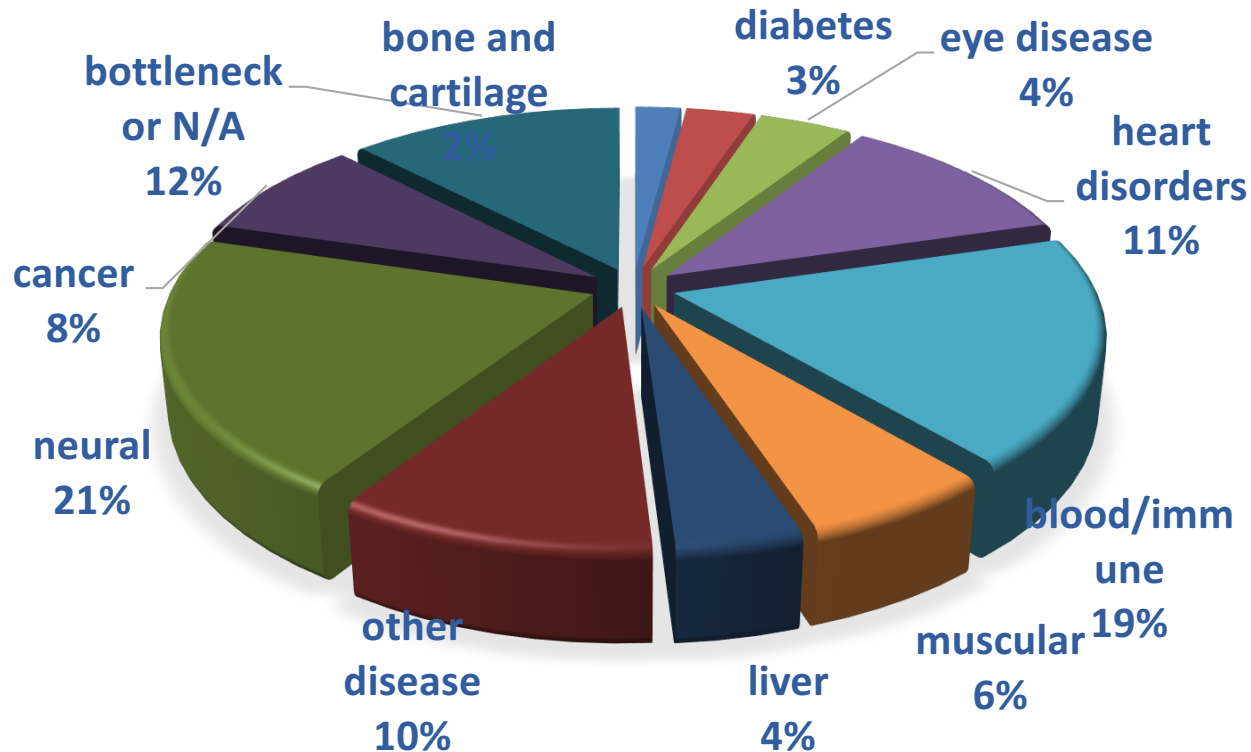
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DISCOVERY

Therapies come from discoveries
100 active discovery projects in CIRM portfolio



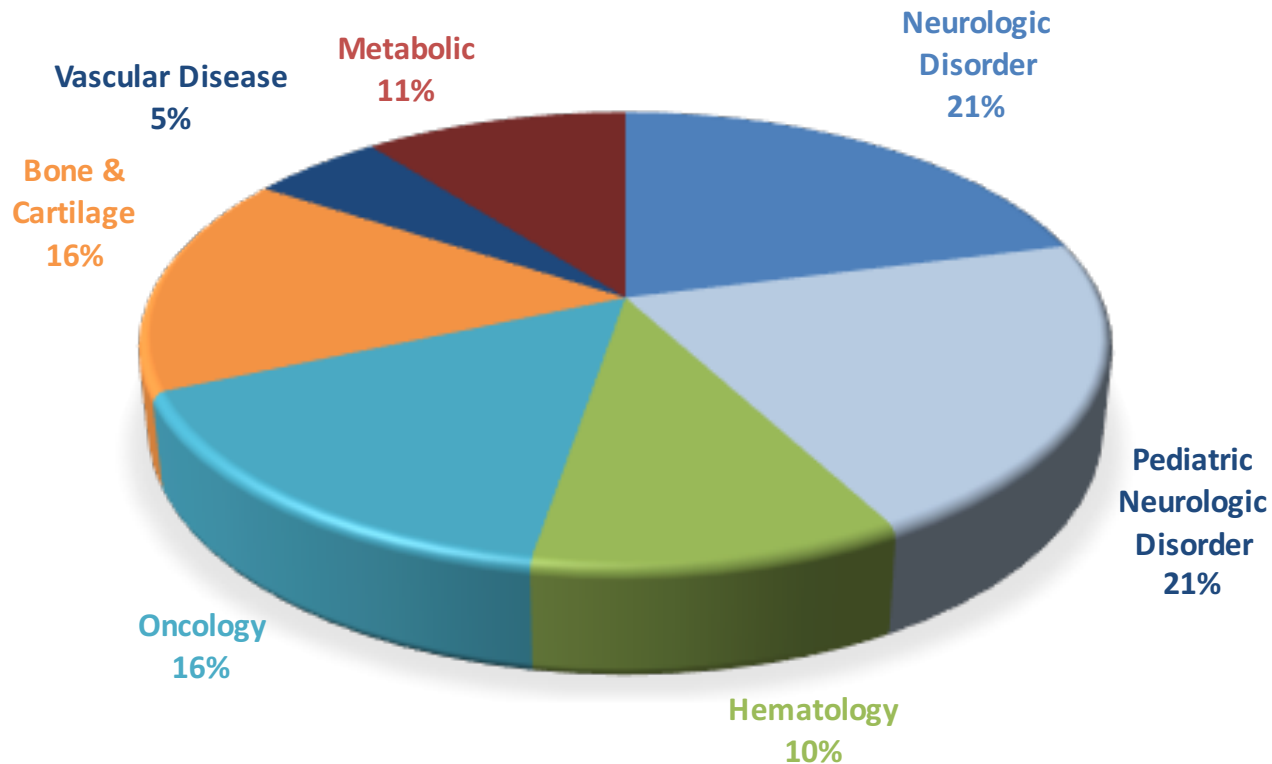
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TRANSLATION

Preparing discoveries for clinical development

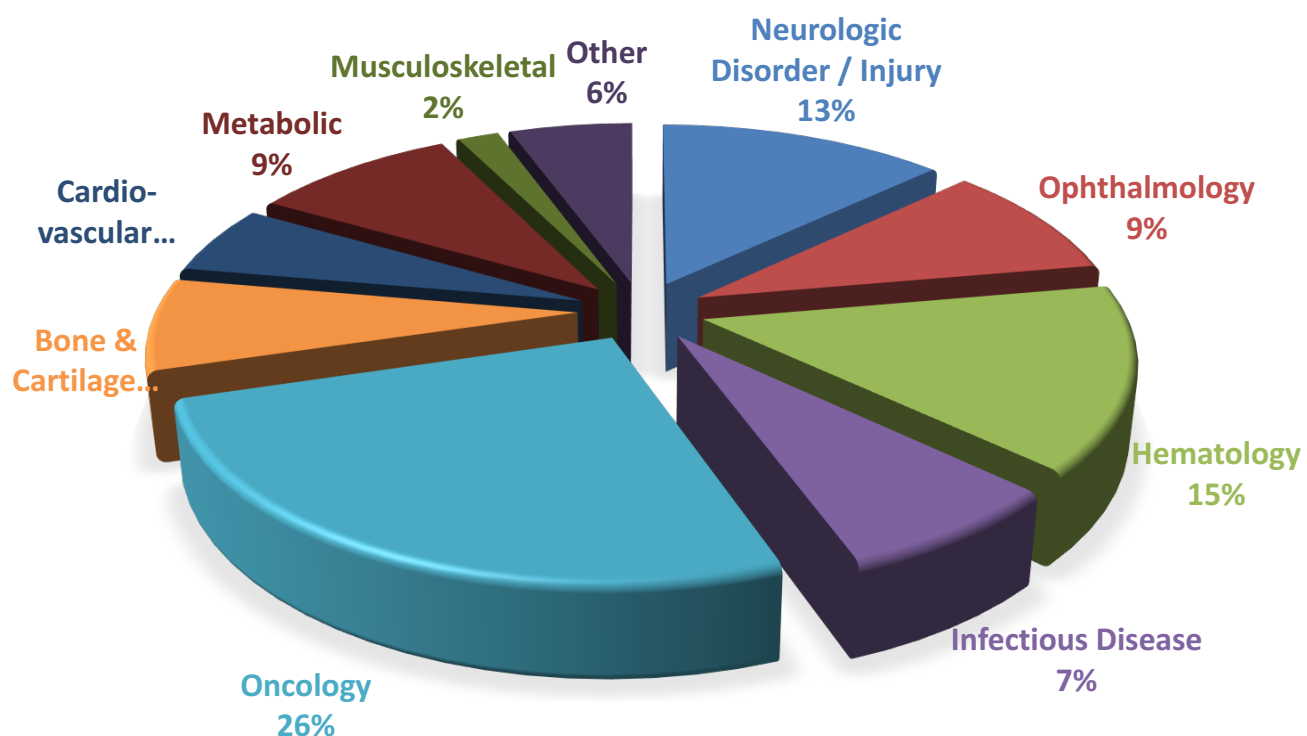
20 active Translational projects in CIRM portfolio





CLINICAL

Accelerating Cures to patients by conducting high quality clinical trials



Phase 1	29
Phase 2	7
Phase 3	5
Other	2
Total	43

CIRM 2.0 Operational Excellence



Active CIRM Engagement

+ Increase in Progression Events

+ Reduced Time to IND

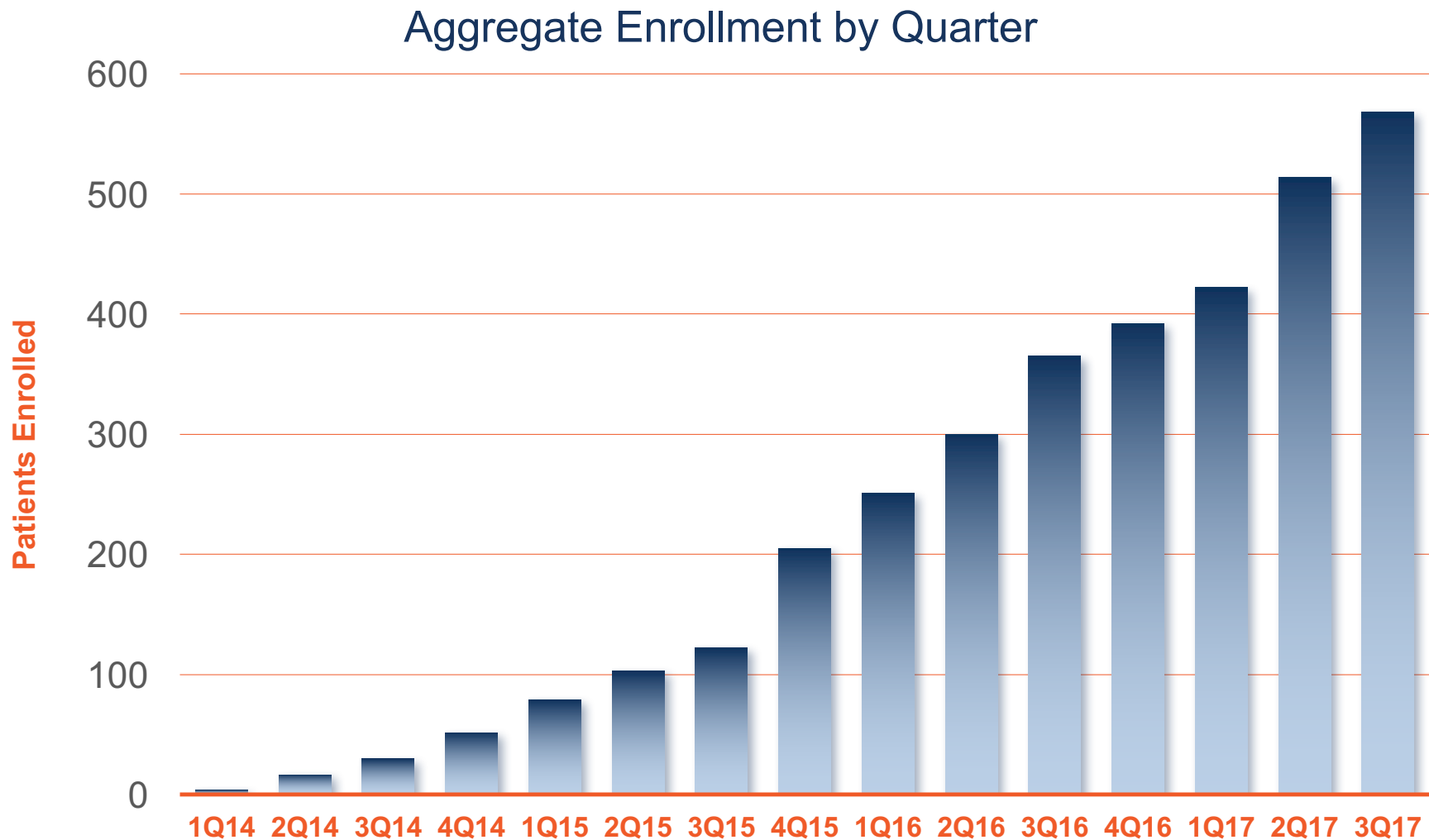
Growth in # of High Quality Clinical Programs



CIRM 2.0 Performance



The system is driving results with our partners

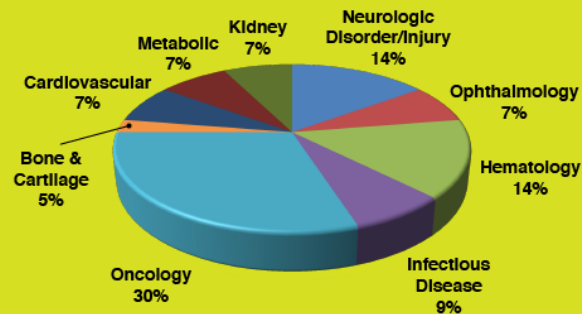


CIRM Clinical Dashboard

CIRM-FUNDED CLINICAL TRIALS:

43

DISEASE AREAS:



LEARN MORE:



Read our CIRM-Funded Trials brochure

All	Blood	Blood Cancers	Bone	Diabetes	Eye	Heart	HIV/AIDS	Kidney	Neuro	Solid Cancers
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<u>Disease Areas</u> ▲	<u>Investigator</u>	<u>Organization</u>	<u>Phase</u>	<u>Trial Status</u>	<u>Targeted Enrollment</u>	<u>Detail</u>
Acute Myeloid Leukemia	Colleen Delaney	Nohla Therapeutics Inc	Phase 2	Not yet recruiting	220	➔
Acute Myeloid Leukemia	Mark Chao	Forty Seven Inc.	Phase 1	Not yet recruiting	96	➔
Adenosine deaminase-deficient Severe Combined Immunodeficiency (ADA-SCID)	Donald Kohn	University of California, Los Angeles	Phase 2	Recruiting	10	➔
Age-related macular degeneration	Mark Humayun	University of Southern California	Phase 1	Recruiting	20	➔

Amyotrophic Lateral Sclerosis

CIRM funded research progresses to clinical trial with gene-modified progenitor cells to treat ALS *now it's personal*

Progenitor Cells Secreting GDNF for the Treatment of ALS

Disease Area: Amyotrophic Lateral Sclerosis
Trial Sponsor: Cedars-Sinai Medical Center
Trial Stage: Phase 1/2
Trial Status: Recruiting
Targeted Enrollment: 18
ClinicalTrials.gov ID: NCT02943850

CIRM Awards Funding This Trial

Investigator: Clive Svendsen
Institution: Cedars-Sinai Medical Center
CIRM Grant: DR2A-05320
Award Value: \$16,961,287

Investigator: Clive Svendsen
Institution: Cedars-Sinai Medical Center
CIRM Grant: CLIN2-09284
Award Value: \$6,154,067



Clive Svendsen

Amyotrophic Lateral Sclerosis

CIRM funding partnership & infrastructure brings in multi-center International Trial for Lou Gherig Disease



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

Disease Area:	Amyotrophic Lateral Sclerosis
Investigator:	Ralph Kern
Institution:	BrainStorm Cell Therapeutics
CIRM Grant:	CLIN2-09894
Award Value:	\$15,912,390
Trial Sponsor:	BrainStorm Cell Therapeutics
Trial Stage:	Phase 3
Trial Status:	Recruiting
Targeted Enrollment:	200
ClinicalTrials.gov ID:	NCT03280056



Ralph Kern

Spinal Cord Injury

FDA Expedited RMAT designation during clinical trial
after review of data thus far

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STRONG

Jake Javier

Spinal Cord Injury,
leaving him paralyzed from
the chest down

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Counts**

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Alpha Thalassemia Major

Combining in utero surgery with stem cell treatment for fatal disease

In Utero Hematopoietic Stem Cell Transplantation For The Treatment Of Fetuses With Alpha Thalassemia Major

Disease Area:	Fetal Alpha Thalassemia Major
Investigator:	Tippi MacKenzie
Institution:	University of California, San Francisco
CIRM Grant:	CLIN2-09183
Award Value:	\$10,906,978
Trial Sponsor:	University of California, San Francisco
Trial Stage:	Phase 1
Trial Status:	Recruiting
Targeted Enrollment:	10
ClinicalTrials.gov ID:	NCT02986698



Tippi MacKenzie

Sickle Cell Disease

Gene corrected stem cells for rare blood disorder afflicting African-American citizens



Clinical Trial of Stem Cell Gene Therapy for Sickle Cell Disease

Disease Area:	Sickle Cell Disease
Investigator:	Donald Kohn
Institution:	University of California, Los Angeles
CIRM Grant:	DR3-06945
Award Value:	\$13,145,465
Trial Sponsor:	University of California, Los Angeles
Trial Stage:	Phase 1
Trial Status:	Recruiting
Targeted Enrollment:	6
ClinicalTrials.gov ID:	NCT02247843




Donald Kohn

Proof of Principle

Curing Incurable Diseases

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CURED

Evangelina
Padilla-Vaccaro

Diagnosed at birth with
Severe Combined
Immunodeficiency

Brenden
Whittaker

Diagnosed with
Chronic
Granulomatous
Disease

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Bubble Baby Disease (SCID)



Confirmatory Trial for FDA Marketing Approval *now it's personal*

Efficacy and safety of cryopreserved autologous CD34+ HSC transduced with EFS lentiviral vector encoding for human ADA gene in ADA-SCID subjects

Disease Area:	Adenosine deaminase-deficient Severe Combined Immunodeficiency (ADA-SCID)
Investigator:	Donald Kohn
Institution:	University of California, Los Angeles
CIRM Grant:	CLIN2-09339
Award Value:	\$19,065,745
Trial Sponsor:	University of California, Los Angeles
Trial Stage:	Phase 2
Trial Status:	Recruiting
Targeted Enrollment:	10
ClinicalTrials.gov ID:	NCT02999984



Donald Kohn

Type 1 Diabetes

Rescue insulin producing cells by taming the immune system with stem cells

Phase 2 Safety and Efficacy Study of CLBS03 Autologous T-Regulatory Cells in Adolescents with Recent Onset Type 1 Diabetes Mellitus

Disease Area:	Type 1 diabetes
Investigator:	Douglas Losordo
Institution:	Caladrius Biosciences, Inc.
CIRM Grant:	CLIN2-09730
Award Value:	\$12,211,255
Trial Sponsor:	Caladrius Biosciences
Trial Stage:	Phase 2
Trial Status:	Recruiting
Targeted Enrollment:	111
ClinicalTrials.gov ID:	NCT02691247



Douglas Losordo

Type 1 Diabetes

Replacement therapy with stem cells

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

Disease Area:	Type 1 diabetes
Investigator:	Howard Foyt
Institution:	ViaCyte, Inc.
CIRM Grant:	CLIN2-09672 (Pre-Active)
Award Value:	\$20,000,000
Trial Sponsor:	ViaCyte, Inc.
Trial Stage:	Phase 1/2
Trial Status:	Recruiting
Targeted Enrollment:	55
ClinicalTrials.gov ID:	NCT03163511



Howard Foyt

Brain Cancer

Targeted killing with gene-modified stem CAR-T

Phase I Study of Chimeric Antigen Receptor Engineered Central Memory T cells for the Treatment of Malignant Glioma

Disease Area:	Brain Cancer
Investigator:	Christine Brown
Institution:	City of Hope
CIRM Grant:	CLIN2-10248 (Pre-Active)
Award Value:	\$12,753,854
Trial Sponsor:	Beckman Research Institute of City of Hope
Trial Stage:	Phase 1
Trial Status:	Recruiting
Targeted Enrollment:	100
ClinicalTrials.gov ID:	NCT02208362



Christine Brown

Blinding Eye Disease



CIRM funded Phase 1 trial yielded data to support CIRM funded Phase 2 trial and FDA expedited RMAT designation

A photograph of a woman with long dark hair, smiling and sitting outdoors. She has sunglasses perched on her head. The background is a blurred blue and green outdoor setting.

VISION

“My dream was to see my kids.
I always saw them with my heart
but now I can see them with
my eyes. Seeing their faces...
is truly a miracle.”

Rosa Benito

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Read Their Stories

2016 Annual Report

Current Funding Opportunities

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Questions?

