



CIRM

CALIFORNIA'S STEM CELL AGENCY

TRANSFORMING
medicine
lives
futures

Maria T. Millan, M.D.

President & CEO
California Institute for Regenerative Medicine

President's Report: June 28, 2018

A young boy with dark hair is shown in profile, looking out a window. He has a gentle smile and his eyes are closed or looking down. The window looks out onto a vast, green, hilly landscape under a bright sky. The scene is peaceful and hopeful.

CIRM's Mission

To accelerate stem cell
treatments to patients with
unmet medical needs

5-yr Strategic Plan

2018 Year 3 Mid-year Update



DISCOVER
50 NEW
CANDIDATES

5

New Candidates



ADVANCE
THROUGH
DEVELOPMENT

3

Progression Events



REFINE
REGULATORY
PATHWAY

1

RMAT



SHORTEN TIME TO
CLINICAL TESTING

pending

IND in 18 months



EXPAND
50 NEW TRIALS
IN 5 YEARS

6

New Trials



INCREASE
INDUSTRY
PULL

6

Partner Events

TOTAL: 29

50

4

3

34

19

Every Moment Counts | Don't Stop Now

CIRM Clinical Trials:

Robust Portfolio

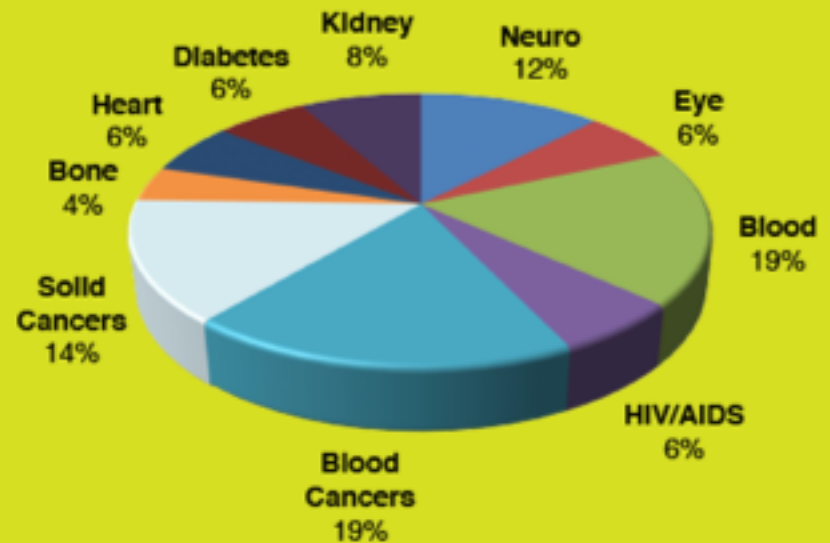
Broad Disease Indications

Diverse Therapeutic Approaches

CIRM-FUNDED CLINICAL TRIALS:



DISEASE AREAS:

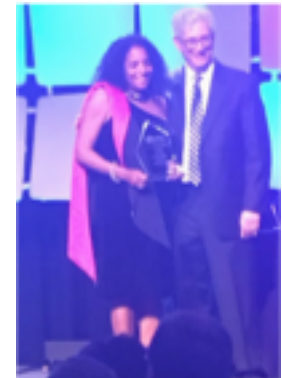


CIRM's Impact is Nationwide

Facilitate & World Stem Cell Summit February 2018



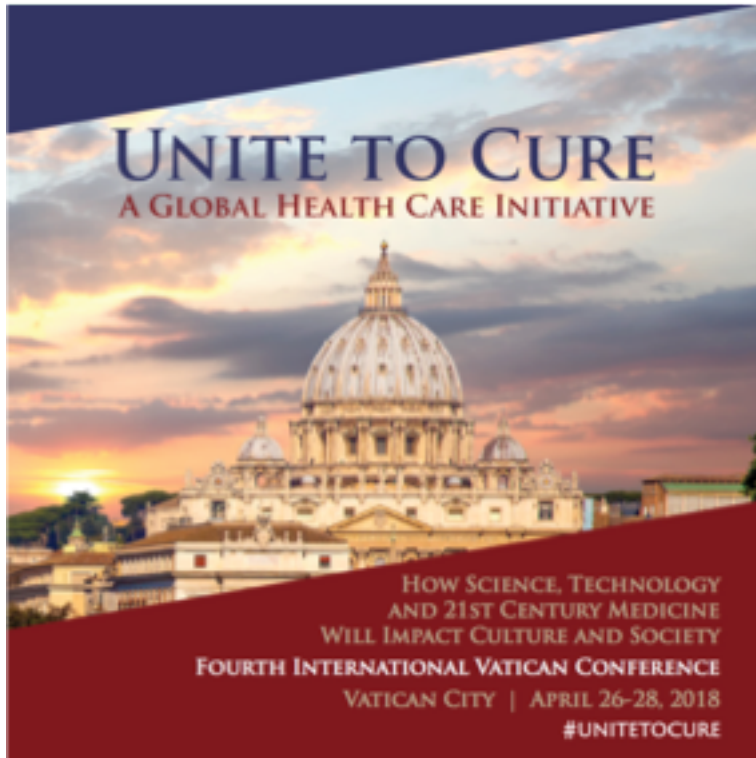
- Institutional Wisdom:
 - Opening Plenary: Clinical Horizons
 - State funding model
 - The Year of Partnership
 - Accelerated Regulatory Pathway
- Leadership
 - Other States and organizations look to us
- Acceleration Model
- Patient Advocacy



CIRM's Mission is Global

Vatican Conference

April 2018



- International Faith, Science, Medicine, Media, Policy & Patient Leaders
- Shared vision to drive cures
- Updates on advances in stem cell/regenerative medicine
- CIRM model for partnership & acceleration
- Pope Francis address:
“Advances in cellular research and in the field of regenerative medicine have opened new horizons...” (*Vatican News*)

“I encourage you, then, to pursue with boldness and determination the ideals that have brought you together” (Zenit)

Every Moment Counts | Don't Stop Now

Collaboration Broadens our Impact



Partnership to Cure Sickle Cell




- Recognizes the value of CIRM's Processes and Funding Infrastructure
- Leverages CIRM application structure and GWG process. NHLBI will use CIRM application to make their funding decisions within CIRM funding timelines
- Co-funding by the NHLBI will allow CIRM to “stretch” dollars and maximize the impact of its research funds in the area of sickle cell disease
- CIRM will contract and manage awards utilizing milestone-based payments
- MOU put in place to work out logistics and processes for this partnership

The CIRM Team:

Our “Intel Inside”

- Gabe Thompson- Operational Excellence
 - Abba Creasey- Expedited Regulatory Paradigm
 - Neil Littman- Industry Alliance
 - Kevin McCormack- Communicating our Story
 - Pat Olson- Translational Advisory Panel
 - Scott Tocher- Lease Update
-
- Chila Silva-Martin- Administrative Budget (Action Item)
 - Gil Sambrano- Review Process and Programmatic Tools (Possible Action)

Because we know what's possible...



CURED

Evangelina
Padilla-Vaccaro

Diagnosed at birth with
Severe Combined
Immunodeficiency

Brenden
Whittaker

Diagnosed with
Chronic
Granulomatous
Disease

**Every Moment
Counts**

CIRM
CALIFORNIA / STEM CELL AGENCY

Every Moment Counts | Don't Stop Now

Operational Excellence

Gabe Thompson

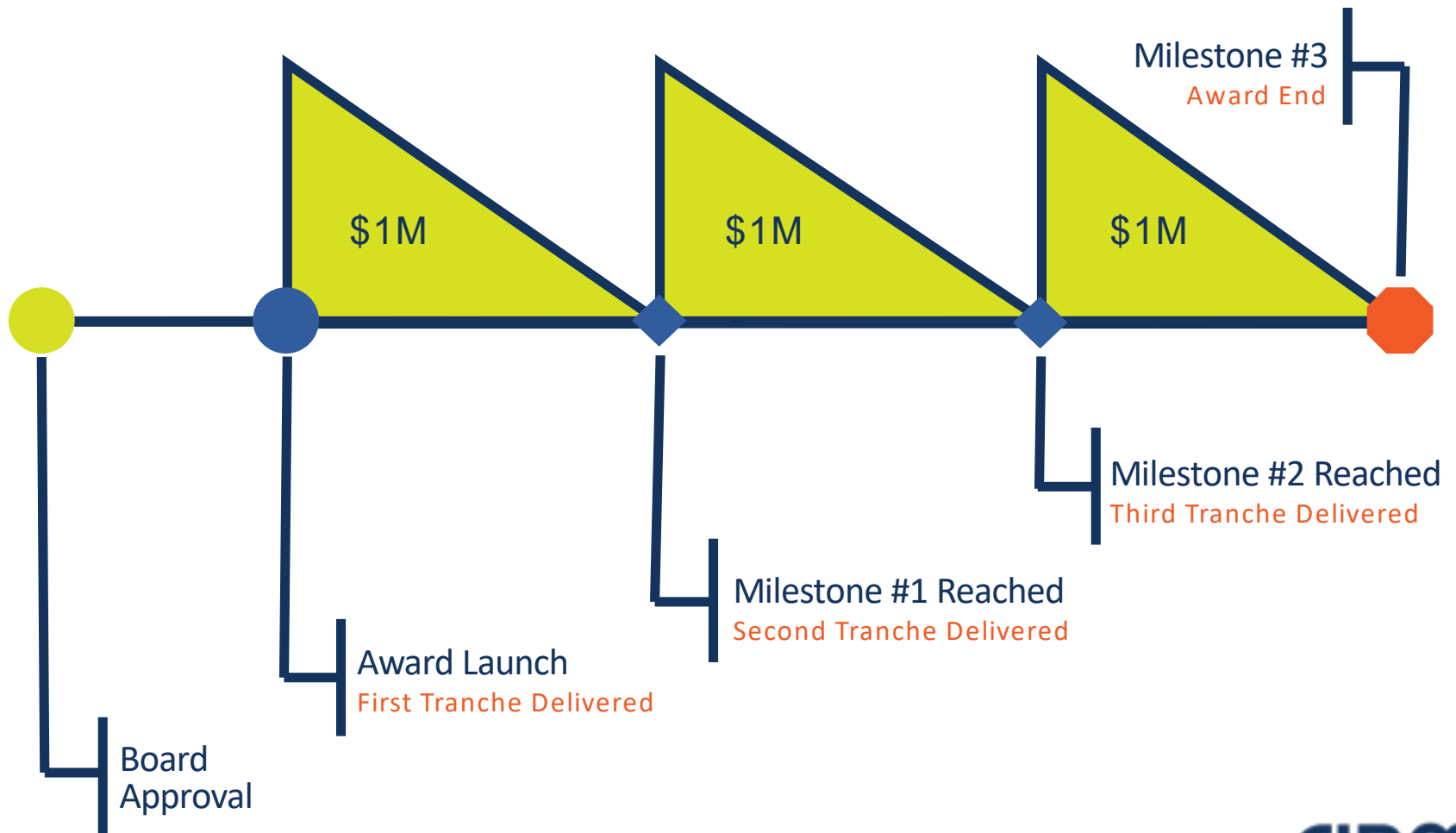
Vice President, grants & Operations
California Institute for Regenerative Medicine

Measuring Acceleration

- CIRM's Operational Milestone Model
- Driving Behavior
- Aggregate Enrollment Rate
- Reducing Administrative Time per Award

Operational Milestones

Disbursements are made upon achieving the milestones



Driving Behavior

Real-time Course Correction

- Advisory Panels
- Project Management

Non-CIRM Contingency Funding

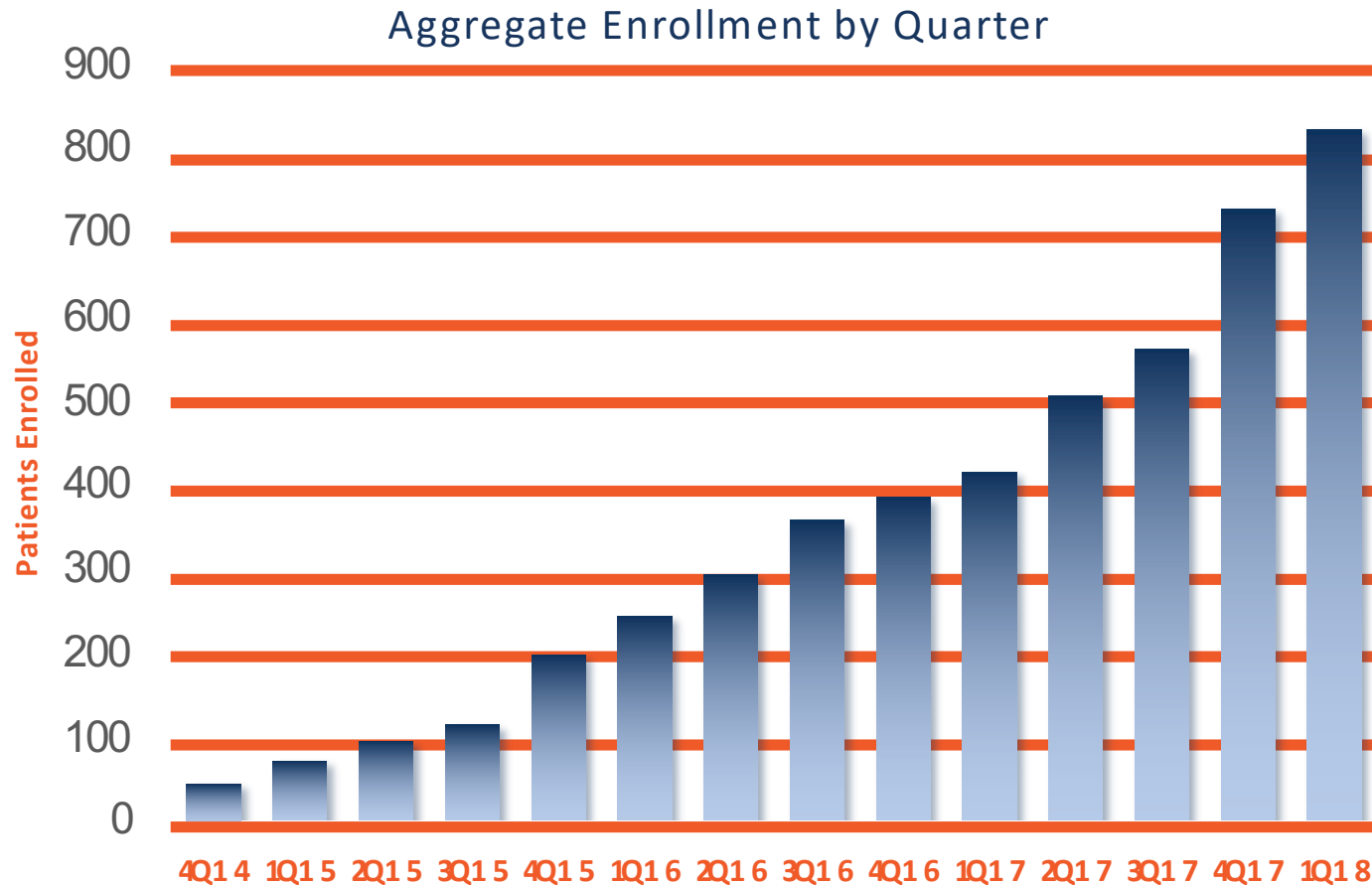
- \$24M provided to date

Award Terminations

Award Type	Awards to Date	Terminations to Date	Awards Amount Returned
CLIN & TRAN	62	4	\$34,140,578

Aggregate Enrollment Update

Enrollment rate of CIRM-funded patients continues to climb



Other Recent Improvements

Award Management

- Implemented **DocuSign** electronic signature of award documents
 - ✓ Average # Days to Execution went from **16 days to 5 days**
- Awardee Payments via **Electronic Funds Transfer**
 - ✓ Payment Processing Time went from **16 days to 4 days**
 - ✓ Reduced mailing costs and payment errors



21st Century Cures

Accelerated Regulatory Pathway

Abla Creasey, Ph.D.

Vice President, Therapeutics & Infrastructure
California Institute for Regenerative Medicine

21st Century Cures Act

December 13, 2016



www.fda.gov



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21st Century Cures Act

Definition of Regenerative Medicine Therapy

- Cell therapy
- Therapeutic tissue engineering product
- Human cell & tissue product
- Combination product

21st Century Cures Act

Regenerative Medicine Advanced Therapy (RMAT) Designation

Special program to advance the field by providing an accelerated pathway to approval for

- Regenerative medicine therapies
- Drugs targeting serious diseases or conditions
- Addresses an unmet medical need

Process for RMAT Designation



60
Days

- FDA determines if RMAT criteria are met in 60 days
- Provides written response of approval
- Provides written rationale if RMAT not awarded

Benefits of RMAT Designation

- Increased access to FDA during early development
- Eligible for priority review and accelerated approval
- Same benefits as FDA fast track and breakthrough designation

Accelerated Approval for RMATs

RMAT therapies with accelerated approval can meet post-approval requirements by:

- Further clinical studies
- Submitting clinical evidence
- Monitoring patient outcomes

Goal: Faster development of stem cell therapies while maintaining standards for safety and efficacy

18 RMAT Designations in 2017 & 2018

Company	Product	Indication	year
Asterias	AST-OPC1	Spinal Cord Injury	2017
Athersys	MultiStem	Ischemic Stroke	2017
Bluebird Bio	LentiGlobin	Severe Sickle Cell Disease	2017
Cellvation	CEVA101	Traumatic Brain Injury	2017
Humacyte	Humacyl	Vascular Access for Hemodialysis	2017
Enzyvant	RVT-802	DiGeorge Syndrome	2017
jCyte	jCell	Retinitis Pigmentosa	2017
Juno	JCAR017	Lymphoma (large B cell NHL)	2017
Kiadis	ATIR101	Leukemia	2017
Mallinckrodt	Stratagraft	Burns	2017
Mesoblast	MPC-150-IM	Heart Failure	2017
Vericel	Ixmyelocel	Dilated Cardiomyopathy	2017
Abeona	EB-101	Recessive RDEB	2018
Abeona	ABO-102	Sanfilippo Syndrome Type A (MPS IIIA)	2018
Capricor	CAP-1001	Duchenne Muscular Dystrophy	2018
MiMedx	AmnioFix Injectable	Osteoarthritis (OA) of the Knee	2018
Nightstar	NSR-REP1	Choroideremia (progressive vision loss)	2018
Caladrius	CLBS	Refractory Angina	2018

Acknowledgements

- Dr. Wilson Bryan – FDA
- CIRM Grantees
 - Asterias
 - Humacyte
 - jCyte
 - Capricor
 - Caladrius

Industry Alliance

Neil Littman

Director, Business Development
California Institute for Regenerative Medicine

Overview

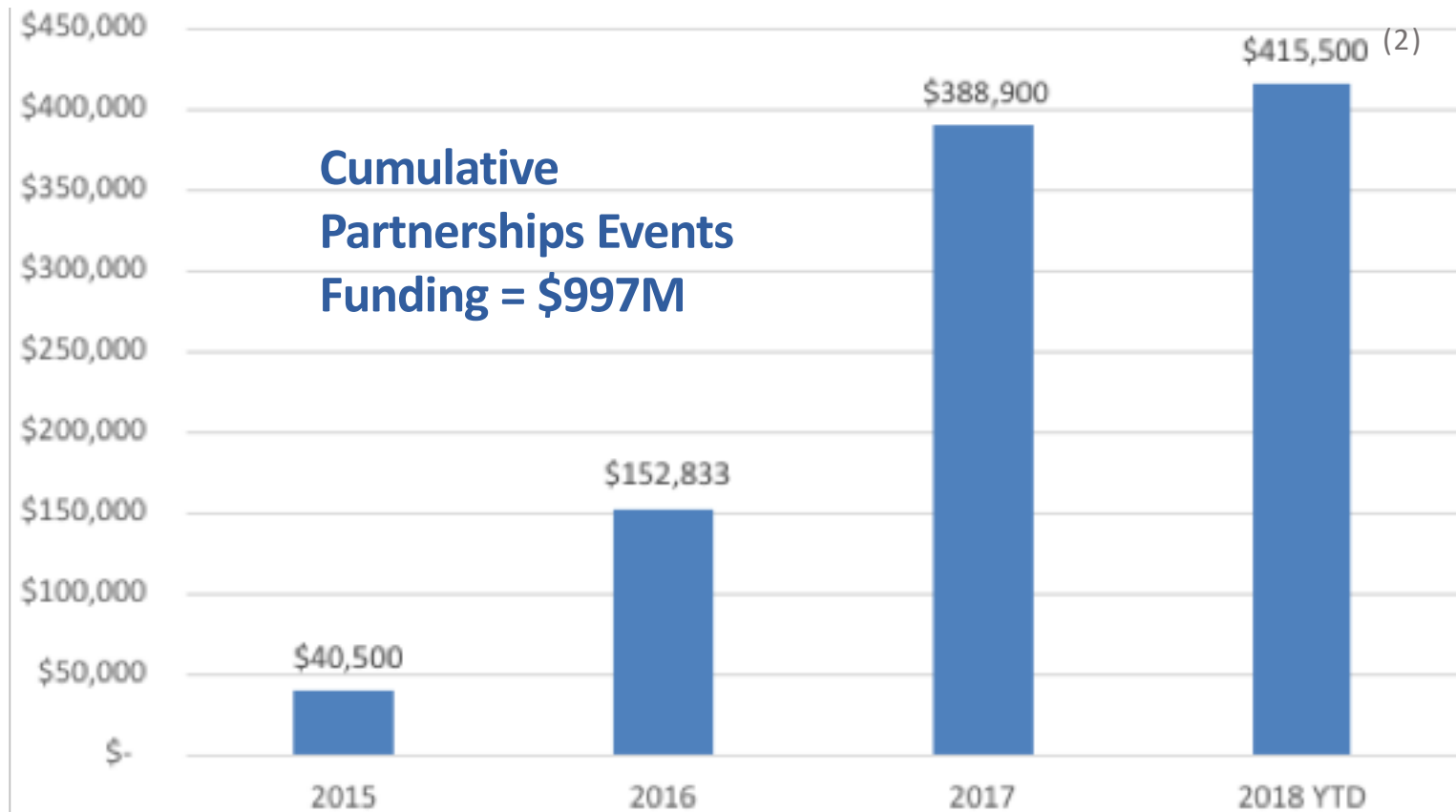


- **Industry Alliance Program** launched in January 2018
- Goal: **Secure industry partnerships and funding** for CIRM's translational and clinical-stage projects
- CIRM is **continuing to selectively recruit new partners** with the goal of **building a collaborative network** to support the development and commercialization of CIRM-funded programs



PANACEA VENTURE







Leveraged Funds: Partnership Events⁽¹⁾



(1) Includes: license agreements, option agreements, and follow-on funding from industry partners and investors.

(2) Includes Forty Seven, Inc. proposed IPO of \$115 million.

2018 YTD Partnership Events

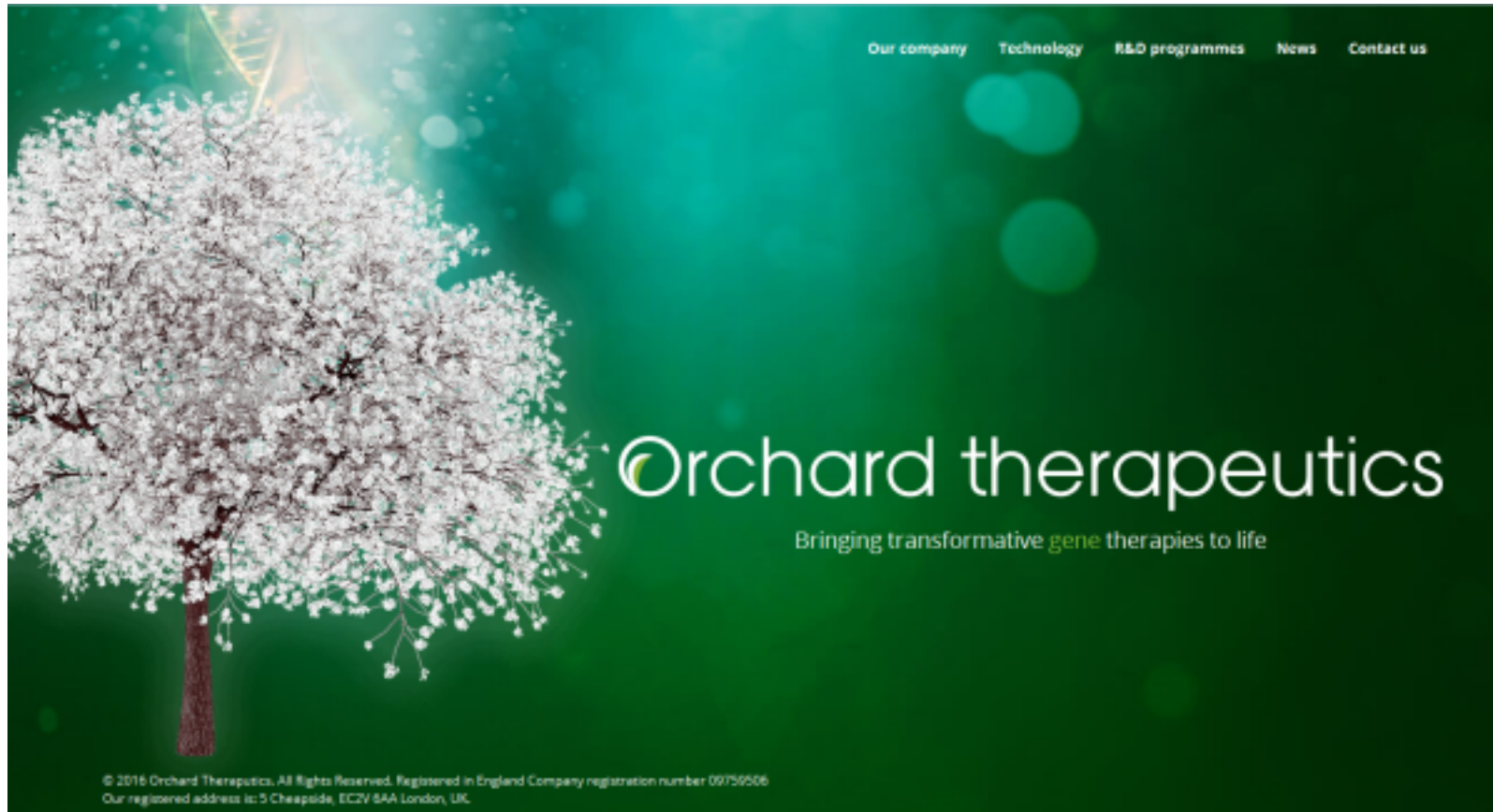
Awardee	Disease Area	Amount
 HUMACYTE [®]	Kidney failure	\$225M
 FORTY SEVEN	Cancer (AML & Colon)	\$115M (IPO filed, not priced)
 NOHLA THERAPEUTICS	Cancer (AML)	\$45M
 POSEIDA THERAPEUTICS	Cancer (Multiple Myeloma)	\$30.5M
 Orchard therapeutics	Rare diseases	Acquired rare disease gene therapy portfolio from  gsk GlaxoSmithKline

Communicating CIRM's Story

Kevin McCormack

Sr. Director Communications & Outreach
California Institute for Regenerative Medicine

Communications Strategy



Media Coverage Clinical Trials

Five Blood Transfusions, One Bone Marrow Transplan
All Before Birth



**The
New York
Times**

**San Jose
Mercury News**

CIRM in the Media



Featured on:



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Facebook Live – Ask the Expert

Stem Cell Therapy for Stroke

Got questions about the progress of
stem cell therapies for stroke?

Then “ASK THE EXPERT”
on **FACEBOOK LIVE**



GARY STEINBERG, MD, PhD
Chair, Neurosurgery
Stanford University



LILA COLLINS, PHD
Senior Science Officer
CIRM

**Thursday May 31st,
Noon to 1pm (PDT)**



Hosted by
CIRM

CALIFORNIA'S STEM CELL AGENCY

 **@CalifornialnstituteForRegenerativeMedicine**

Facebook Live – Ask the Expert - Stroke

91 peak live viewers

6,750 video views

Top audience age group: 45 – 54 years

Top Viewing Locations

1) California – 24%

2) Tokyo – 7.88%

3) New York – 3.67%

Patient Advocacy



Our Mission

Accelerate Stem Cell Treatments
To Patients with
Unmet medical needs.

Clinical Trials

Funding Opportunities

www.cirm.ca.gov

Translational Advisory Panel

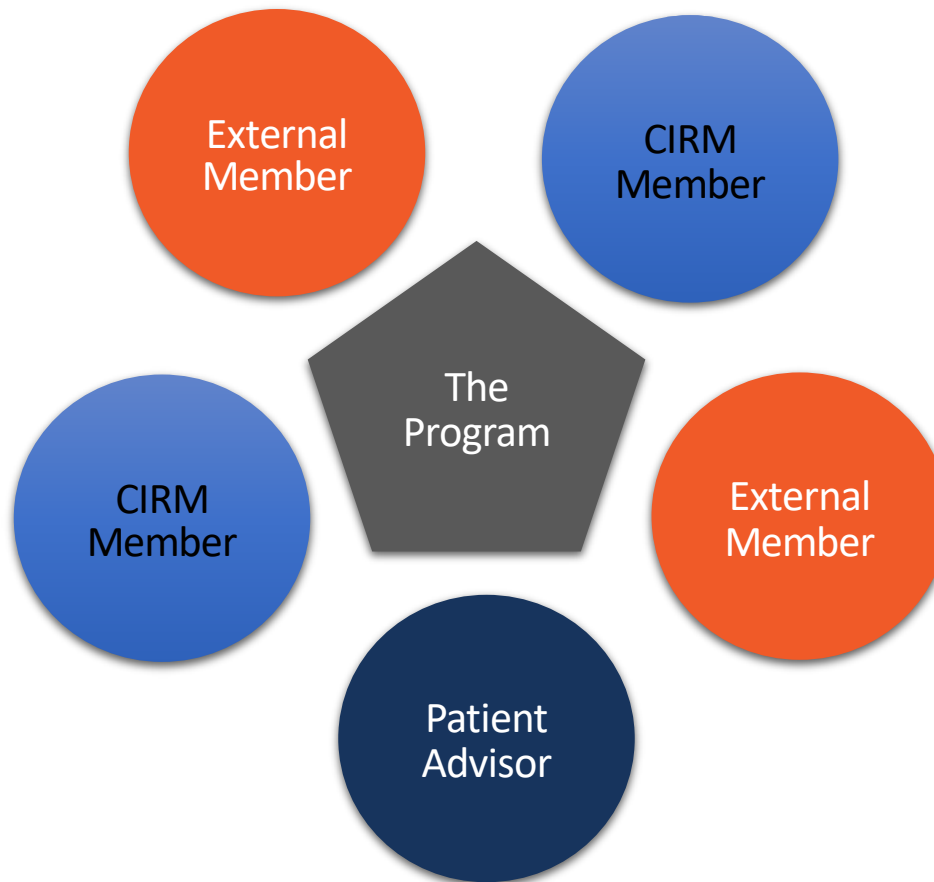
Patricia Olson, Ph.D.

Vice President, Discovery & Translation
California Institute for Regenerative Medicine

Translational Advisory Panel (TAP)

- **Problem:** Translational research is inherently challenging, i.e. process development and cell production
- **Goal:** To accelerate timelines, increase the probability of meeting milestones on time and increase the chances of progressing to a CLIN stage program
- **Solution:** Modeled after the Clinical Advisory Panel, TAPs will increase the expertise available to the project team to solve issues

TAP Membership



Translational Advisory Panel: Status

- Administrative, legal, IT infrastructure in place
- First 2 TAP meetings scheduled for July
 - Scientific and patients advisors recruited
- Planning underway for further TAPs
- Responsible
 - Program Lead: Kent Fitzgerald
 - Program Logistics: Amy Cheung