

SOMETHING  
BETTER  
THAN HOPE Right now.

CIRM Update to CFAOC  
August 23, 2019

Maria T. Millan, MD  
President & CEO



Every Moment Counts. **Don't Stop** Now.

**Our Mission**  
Accelerate Stem Cell Treatments  
To Patients with  
Unmet medical needs.



**2004**

CIRM created by Patient Advocates and California Stakeholders-Proposition 71

**\$3B**

Committed to CIRM Mission

**1000 (200 UNDER ACTIVE MANAGEMENT)**

Cutting Edge Research & Transformative Programs funded

**56 CLINICAL TRIALS**

First in human, cell & gene medicine, some ready for final marketing approval

**>1200 PATIENTS**

Patients enrolled in CIRM Funded Clinical Trials

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**CIRM**  
CALIFORNIA'S STEM CELL AGENCY

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# CIRM Investments



INFRASTRUCTURE

\$482M



EDUCATION

\$219M



DISCOVERY

\$904M



TRANSLATION

\$329M



CLINICAL

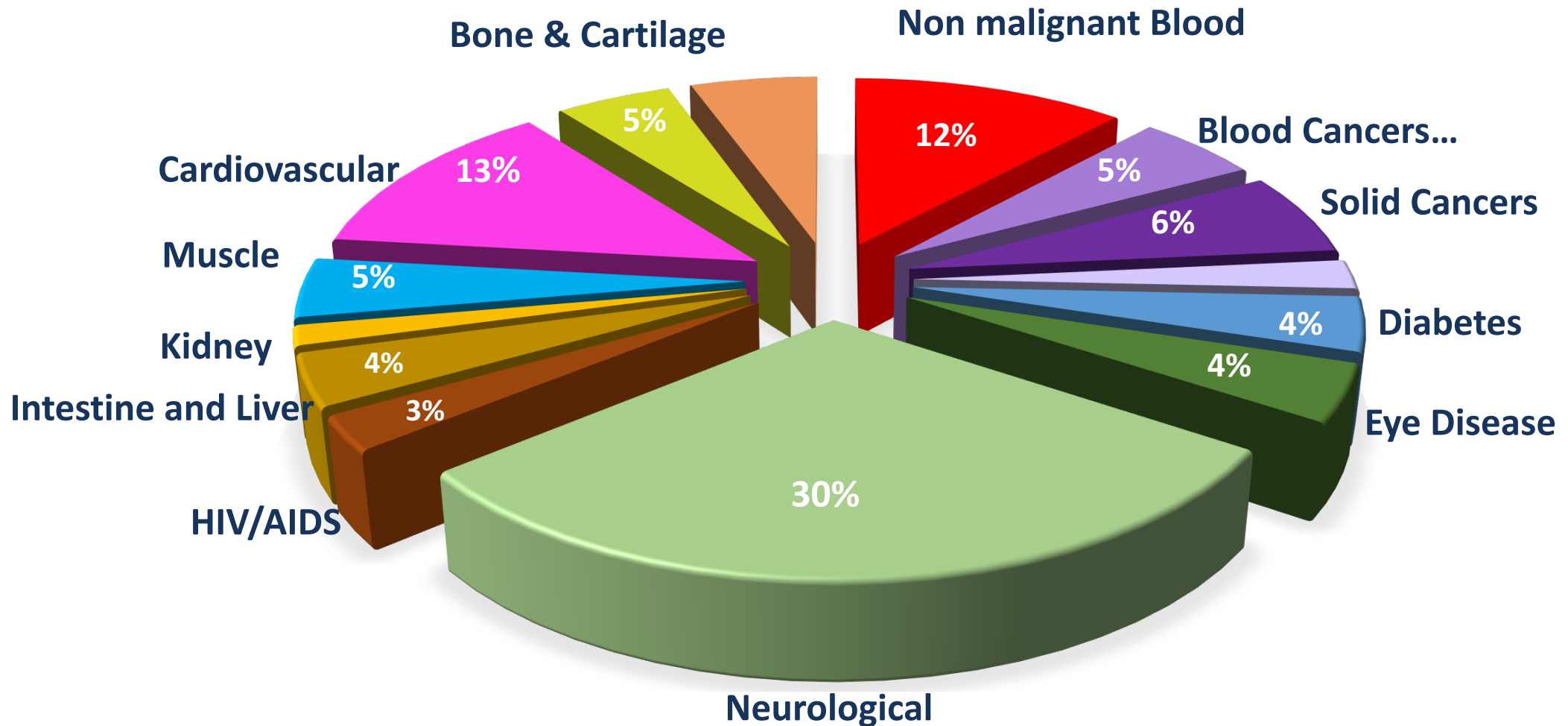
\$708M

**\$2.642B > 1000 Awards**

**\$1.9B for Therapeutic Research & Development**

*Through 6/30/2019*

# CIRM Research & Development Portfolio





# 56 Clinical Trials: To Tackle Over 35 Different Unmet Medical Needs

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

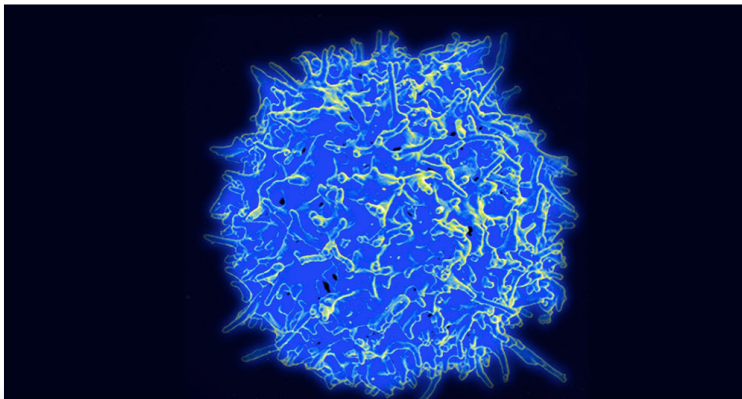
Huntington's Disease  
Kidney Failure  
Lung Cancer  
Melanoma  
Multiple Myeloma  
Bone Disease  
Immune Deficiencies  
Sickle Cell  
Metastatic Cancer  
Paralysis  
Stroke  
Diabetes

# Paradigm Shift with Cell and Gene Therapy

## Precision Medicine & Curative Treatment

Medicare proposes a boost in payments for CAR-T and other breakthrough drugs and devices

By CASEY ROSS @caseymross / APRIL 23, 2019



5 CIRM CAR-T clinical programs (\$57 M) for:

- Brain Cancer- Glioblastoma
- Multiple Myeloma
- B cell malignancies
- Breast cancer metastatic to brain
- Prostate cancer
- Other solid Cancers

*Patient's own  
immune cells  
engineered to  
target & kill cancer*

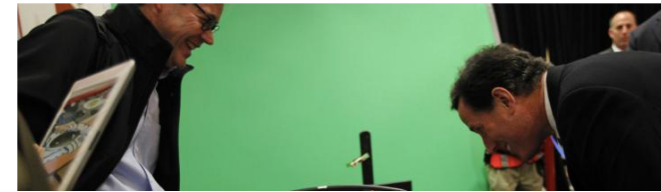
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### TECH & SCIENCE

## THE MOST EXPENSIVE DRUG IN THE WORLD IS ABOUT TO BE MADE WORTHLESS BY GENE THERAPY

BY KATE SHERIDAN ON 11/1/17 AT 5:46 PM EDT



25 CIRM (\$235 M) gene programs for:

- Cancer
- HIV/AIDS
- Lysosomal Storage Disease
- Primary Immune Deficiencies
- Sickle Cell Disease
- Beta Thalassemia
- Granulomatous Disease
- Neurologic Diseases

*Modify patient's  
genes to treat or  
cure a disease*

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# Paradigm Shift with Cell and Gene Therapy

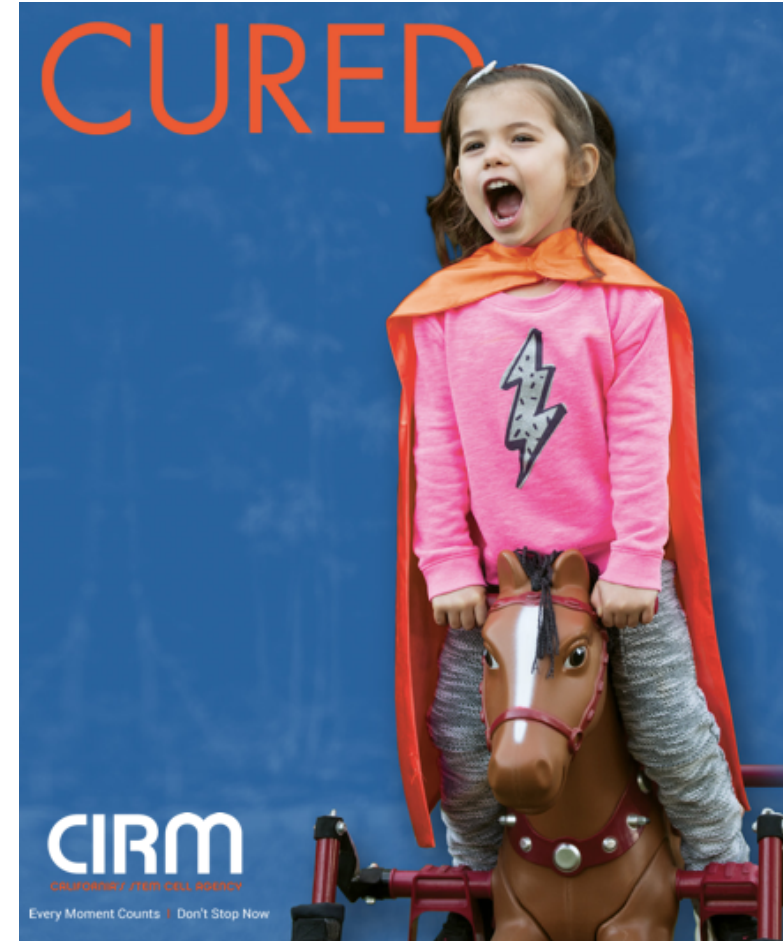
The beginning of a new era  
in cell & gene medicine...

Gene correction in inherited fatal immune  
deficiency has resulted in cures

Dr. Don Kohn (UCLA) and Orchard Therapeutics  
are completing the pivotal trial- FDA marketing  
approval application in 2020

As a viable therapeutic approach, it marks the  
beginning for potential cures for  
7000 currently untreatable genetic diseases

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Evie is more than 5 years from treatment in a  
clinical trial as an infant. She is durably cured  
as are >30 others.

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# CIRM Partnership to Cure Sickle Cell Disease



- CIRM and NHLBI MOU to jointly fund industry and academic cell and gene programs for the Cure Sickle Cell Initiative
- Leverages CIRM's Processes and Funding Infrastructure
- American Society of Hematology setting up registry & data capacity

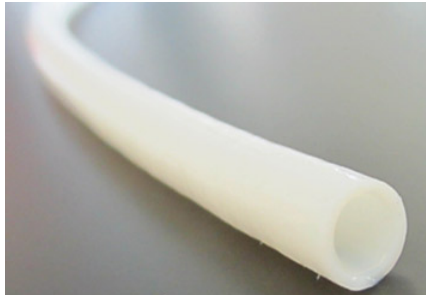


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# CIRM targets large patient populations:

## Tissue Engineered Vessel for Kidney Failure



**HUMACYTE**<sup>®</sup>



190,000+  
U.S PATIENTS  
SERVED



2,200+  
U.S DIALYSIS CLINICS



26M  
ANNUAL  
HEMODIALYSIS  
TREATMENT  
EQUIVALENTS IN  
NORTH AMERICA

PRE-CLINICAL

PHASE I

PHASE II

PHASE III

LAUNCH

A-V (VS. PTFE)

A-V (VS. FISTULA)

PAD EU

PAD US

VASCULAR TRAUMA

} **\$24M CIRM Funding**



**FRESENIUS  
MEDICAL CARE**

\$150M Equity Investment  
Global marketing,  
sales/distribution

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# CIRM Clinical Portfolio: Leveraged Funding

CIRM funding de-risks programs so they can gain early data to obtain additional investments.

\$2.6 B in CIRM funding leveraged  
\$3.3B additional funding into CIRM programs:

Industry partnerships \$1.82B  
co-funding \$1.02B  
grants and gifts \$489M

Industry Partnership  
>\$1.82 Billion  
Total YTD 2019

2018 | \$1.06 Billion

2017 | \$389 M

2016 | \$153 M

2015 | \$40.5 M



# Partnering Events

2015

2016

2017

2018



cancer



eye disease



bone healing



neurological



cancer



rare diseases



heart disease



cancer



genetic disorder



HIV/AIDS



eye disease



cancer



rare disease



cancer



cancer



kidney failure



leukemia



spinal cord injury



Type 1 Diabetes



pediatric immunodeficiency



cancer



cancer

*\*includes licenses, options, and additional funding*

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# CIRM : A Hub to Promote Partnership

Patient Advocates



Consortia & Partners



CIRM Researchers



*Credit for image: SciHub Copernicus*

Investors



Alpha Clinics Network



Hub for Interactions with and between grantees, non-profit Organizations, the Public & Industry

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**Thank You!**