

# Driving Standards for Regenerative Medicine Treatments in California: A Perspective from CIRM

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## CIRM Background

### 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**

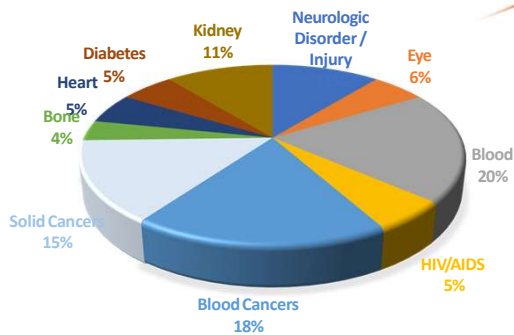
Patients enrolled in CIRM Funded Clinical Trials

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## 56 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

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## Presentation Outline

- **State of the Field:**  
Overview of regenerative medicine treatments making their way to patient
- **Risks to Patients of Unregulated Treatments:**  
Harms resulting from unproven stem cell treatments
- **Standards:**  
Regulated, Reputable and Reliable
- **A Path Forward:**  
Considerations for patient protection

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# State of the Field Globally

**1,069**

Clinical Trials Underway  
Worldwide by End of Q2 2019

**Ph. I: 358**  
**Ph. II: 617**  
**Ph. III: 94**

## Number of Clinical Trials Utilizing Specific RM/AT Technology: Q2 2019



### Gene Therapy

**Total: 366**  
Ph. I: 117  
Ph. II: 219  
Ph. III: 30



### Gene-Modified Cell Therapy

**Total: 410**  
Ph. I: 187  
Ph. II: 207  
Ph. III: 16



### Cell Therapy

**Total: 249**  
Ph. I: 49  
Ph. II: 168  
Ph. III: 32



### Tissue Engineering

**Total: 44**  
Ph. I: 5  
Ph. II: 23  
Ph. III: 16

Source: <https://alliancerm.org>

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# Unregulated Treatments Pose Risks

News & Analysis

Medical News & Perspectives

Unproven but P

Rita Rubin, MA

**FDA warns StemGenex Biologic Laboratories LLC of illegally marketing an unapproved cellular**

**produ**  
**significa**

**FTC Stops Deceptive Health Claims by a Stem Cell Therapy Clinic**

**Defendants lacked scientific evidence that their "amniotic stem cell therapy" could treat or cure serious diseases, including Parkinson's, macular degeneration, cerebral palsy, and autism**

THE NEW ENGLAND JOURNAL OF MEDICINE

BRIEF REPORT

**Vision Loss after Intravitreal Injection of Autologous "Stem Cells" for AMD**

Ajay E. Kuriyan, M.D., Thomas A. Albin, M.D., Justin H. Townsend, M.D.,  
Marianelli Rodriguez, M.D., Ph.D., Hemang K. Pandya, M.D.,  
Robert E. Leonard II, M.D., M. Brandon Parrott, M.D., Ph.D.,  
Phillip J. Rosenfeld, M.D., Ph.D., Harry W. Flynn, Jr., M.D.,  
and Jeffrey L. Goldberg, M.D., Ph.D.

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## Characteristics and Scope of Training of Clinicians Participating in the US Direct-to-Consumer Marketplace for Unproven Stem Cell Interventions

Table 1. Professional Backgrounds of Physicians and Nonphysicians Providing Stem Cell Interventions in California, Florida, and Texas

Profession	Individuals, No. (%)
<b>Physicians</b>	
Doctor of medicine	338 (55.6)
Doctor of osteopathy	56 (9.2)
Medical degree (eg, MBBS) from non-US schools	7 (1.2)
Total physicians	401 (66.0) <sup>a</sup>
<b>Nonphysicians</b>	
Physician assistants	55 (9.0)
Nurses	34 (5.6)
Complementary and alternative medicine practitioners	32 (5.3)
Podiatrists	29 (4.8)
Physical therapists	18 (3.0)
Other <sup>b</sup>	13 (2.3)
Scientists with a master's degree or doctorate in philosophy	10 (1.6)
Unclear qualifications	10 (1.6)
Dentists	6 (1.0)
Total nonphysician clinicians	207 (34.0)
<b>Total clinicians</b>	<b>608 (100)</b>

<sup>a</sup> Mean age of 55 years (range, 32-97 years). Mean licensed years of practice of 25 years (range, 2-61 years).

<sup>b</sup> Includes respiratory therapists, estheticians, clinical laboratory technicians, athletic trainers, radiology practitioner assistants, and support staff.

- 351 companies at 570 clinics with the largest concentration in CA, FL & TX (*Cell Stem Cell 2016, Turner & Knoepfler*)
- 166 companies, 608 clinicians (66% physicians) in these 3 states in Jan. 2018
- 5 companies staffed exclusively by podiatrists, 2 by naturopaths, 1 by dentists and 1 by practitioners of unclear qualifications
- Of 157 companies with a physician, 52% had 1 physician with formal training to match conditions claimed to treat
- 77% of orthopedic-focused practices had 1 or more physicians with appropriate specialty training
- **Only 19% of companies marketing stem cells for nonorthopedic indications had physicians practicing within their scope**
- **In 2018, the FSMB reported that 17 of 51 boards investigated complains and 8 took disciplinary actions related to physicians performing unlicensed stem cell procedures**

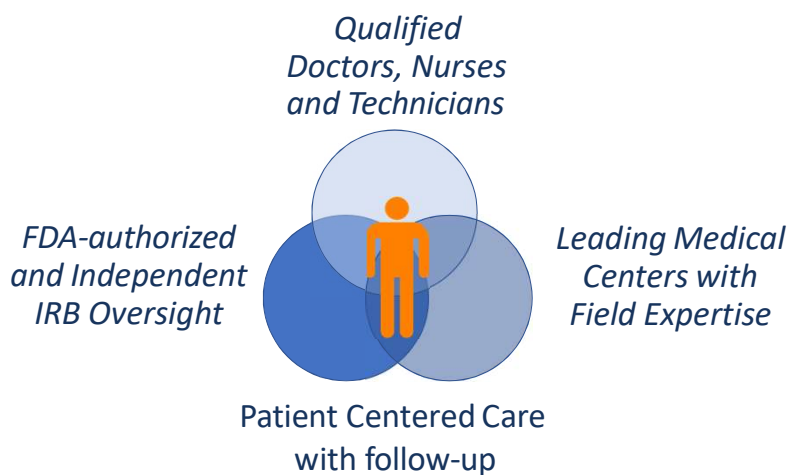
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## Standards for the Delivery of Regenerative Medicine

### Regulated Reputable Reliable



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## What do we Have in Place Today?

- Clinicaltrials.gov – a trial registry being misused by unregulated clinics
- California Senate Bill 512 Dr. Hernandez (2017)- disclosure to patients that they are receiving unregulated putative “stem cell” therapy
- 2017 FDA “Framework for Regenerative Medicine Products” to provide clarity
- ISSCR “A Closer Look at Stem Cells” information and “red flags” for patients
- National Academy of Sciences & WHO convening gap analysis and policy discussions on regulating the stem cell industry

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## Build on Existing CA Policy Framework

- **Senate Bill 512 Dr. Hernandez (2017)**
  - Human cells, tissues or cellular or tissue-based products “HCT/Ps” as defined by FDA
  - If HCT/Ps are not FDA-approved, then patient must be informed of the following:  
*“THIS NOTICE MUST BE PROVIDED TO YOU UNDER CALIFORNIA LAW. This health care practitioner performs one or more stem cell therapies that have not yet been approved by the United States Food and Drug Administration. You are encouraged to consult with your primary care physician prior to undergoing a stem cell therapy.”*
- **Apply consent and professional certification requirements to products and caregivers covered under SB 512**

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## Professional Standards for Patient Disclosure

- **Informed Consent Process**
  - Rationale for providing the treatment and perceived benefits
  - Potential risks or adverse events
  - Oversight and monitoring
  - Cost to patient
  - Qualifications of organization / treatment team
  - Alternatives treatments for this condition
- **Certified Caregivers Providing Consent**

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## For Consideration:

- Publicly available registries of stem cell treatment options
  - *Consider a designated section for patient self-reports from unregulated treatments that is clearly labeled as unregulated*
- Requirement of clear visibility to treatment team's credentials at clinics offering regenerative medicine treatments
- A mechanism for investigating those holding healthcare licenses suspected of violating professional standards when providing unproven stem cell interventions, particularly those outside their scope of training
- Registration of treatments that involve HCT/P's regulated under the PHS Act and clearly distinguish between FDA-authorized treatments and practice of medicine

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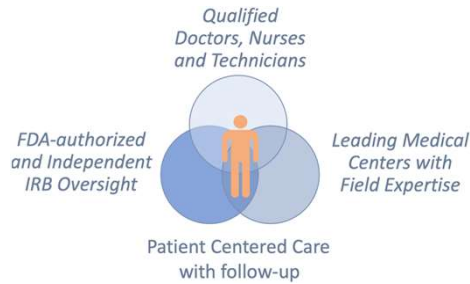
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# Regulated Reputable Reliable

## Can We Leverage Existing Capacity?



- Licensing
- Enforcement
- Outreach



- Alpha Clinics Network and Clinical Sites- Model & Standards creation
- Fellowships in specialty-based regenerative medicine research
- Alignment with FDA
- Patient navigation & education resources
- Potential involvement in registry

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## CIRM: Partnerships & Stakeholders

Patient Advocates



CIRM Researchers



Consortia & Partners



Alpha Stem Cell Clinics Network



Credit for image: Schib Copernicus

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

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## Multi-Stakeholder Effort

### The Washington Post

Health

## New Google policy bars ads for unproven stem cell therapies

"Untested, deceptive treatments" can endanger consumers, Internet giant says.



September 6, 2019

Its new policy will prohibit ads for treatments that have "no established biomedical or scientific basis."

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**Thank You!**

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