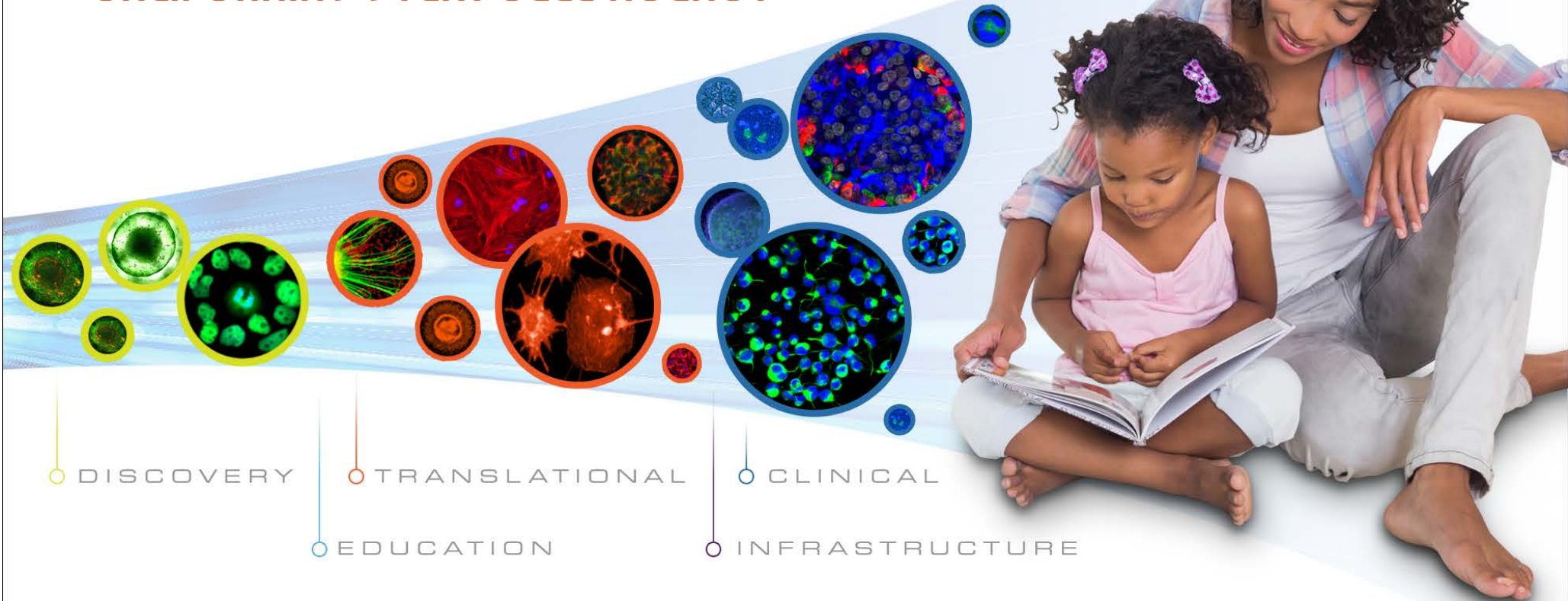


March 2016

# CIRM

CALIFORNIA'S STEM CELL AGENCY



○ DISCOVERY      ○ TRANSLATIONAL      ○ CLINICAL  
○ EDUCATION      ○ INFRASTRUCTURE

**INFR 2 03.21.16: The CIRM Translating Center-  
Stem Cell Focused Preclinical Research Organization**

## Our Mission

To accelerate stem cell treatments to patients with unmet medical needs.

# Challenges to Accelerating Stem Cell Therapies

**Challenge:** Moving stem cell treatments through IND-enabling studies to clinical trials takes disproportionately more time when compared to non-cellular treatments.

**Solution:** As integral components of CIRM's Strategic Plan, CIRM's infrastructure programs are designed to overcome the obstacles that currently slow the progress of translating stem cell research to high quality clinical trials.

see: Strategic Plan @ [www.cirm.ca.gov](http://www.cirm.ca.gov)



# CIRM Infrastructure Programs

| Program                      | Goal                                 | Role  |
|------------------------------|--------------------------------------|---|
| <b>Translating Center</b>    | Shorten time to clinical testing     | <ul style="list-style-type: none"><li>• Process Development</li><li>• IND-enabling activities</li></ul>   |
| <b>Accelerating Center</b>   | Accelerate clinical research         | <ul style="list-style-type: none"><li>• Regulatory Submissions</li><li>• Trial management</li></ul>   |
| <b>Alpha Clinics Network</b> | Conduct high quality clinical trials | <ul style="list-style-type: none"><li>• Specialize in cell therapy clinical trials</li><li>• Develop AVARs (Accelerating and Value Add Resources)</li></ul> |

# Translating Center

**CIRM**<sub>20</sub>  
CALIFORNIA STEM CELL INSTITUTE

INFRASTRUCTURE

Partnering Opportunity  
to Create a CIRM  
Translating Center

INFR 2



REQUEST FOR APPLICATIONS

03.21.16

- CIRM funding for a Stem Cell focused Preclinical Research Organization
- Operating within California
- Up to \$15 million over five years

**CIRM**  
ALPHA STEM CELL CLINICS NETWORK

# Translating Center Core Services

- Consultation services
- Project Management
- Preclinical Development Research
- Process Development
- Product Manufacturing
- Regulatory Filing Support

Services will be proportional to the needs of the projects

# Translating Center Services

- Provide consultation to clients about appropriate process development and preclinical studies
- Conduct preclinical research activities, including pivotal pharmacology and toxicity studies
- Develop cGMP-compatible manufacturing and processes for stem cell and gene modified stem cell products
- Manufacture stem cell and gene modified stem cell products under cGMP
- Generate documentation required for filing regulatory applications

# Sustainability

- Through acquisition of unique insight and experience by supporting CIRM's projects, the TC will be positioned to develop specialized approaches and services for cell therapy trials
- The TC is expected to leverage these assets to create a sustainable platform for support of cell therapy development and stem cell clinical trials



# Award Requirements

- Center Director must commit 100% effort for the first 3 yrs. of CIRM funding and no less than 80% thereafter
- Majority of TC operations involving the use of CIRM funds must take place from a CA-based facility
- The award does NOT support the cost of constructing or renovating facilities

# How Will Applications be Evaluated?

- **Significance and Potential**
  - Is the proposed center likely to accelerate the progression of projects
  - Will it be sustainable
  - How strong are the offerings
- **Strength of the Plan**
  - Strength of resources
  - Does the timeline reflect the “sense of urgency” to address the stated needs
  - Will the center be able to effectively coordinate with the Accelerating Center to support IND submissions
  - Will it effectively/efficiently support CIRM programs while building an overall business enterprise
- **Feasibility**
  - Personnel qualification and track record
  - Staffing & resourcing for the required services
  - Risk mitigation and contingency plans

# Application Process

**Online Application:** Mid April

## **Application Components:**

- Project Summary
- Statement of Significance and Impact
- Project Plan
- Operational Plan
- Business & Sustainability
- Timeline
- Organizational Experience and Assets
- Core Service Capacity, Resources and Environment
- Team Organization
- Contingency Plan
- References

# Process for Funding and Timeline

|  |   |
|--|---|
| <b>Applications Due</b>                  | 2:00 PM PDT, July 15, 2016                  |
| <b>Grants Working Group (GWG) Review</b> | Q3/Q4 2016                                  |
| <b>ICOC Review and Approval</b>          | Q3/Q4 2016                                  |
| <b>Award Start</b>                       | Must start within 45 days of award approval |

# Helpful Contacts and Links

For Application Questions: [GSambrano@cirm.ca.gov](mailto:GSambrano@cirm.ca.gov)

For RFA Questions: [SLin@cirm.ca.gov](mailto:SLin@cirm.ca.gov)

Strategic Plan link: [www.cirm.ca.gov](http://www.cirm.ca.gov)

Alpha Clinics link: <https://www.cirm.ca.gov/patients/alpha-clinics-network/about>

Accelerating Center link: [https://www.cirm.ca.gov/sites/default/files/files/about\\_cirm/INFR1\\_Accelerating\\_03.01.16.pdf](https://www.cirm.ca.gov/sites/default/files/files/about_cirm/INFR1_Accelerating_03.01.16.pdf)