

# **Webinar for potential applicants CIRM Alpha Stem Cell Clinics (CASC) Network**

Alpha Stem Cell Clinic Award RFA 13-06  
Coordinating and Information Management Center RFA 13-07

Maria T. Millan, MD, Medical Officer – RFA 13-06

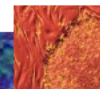
Natalie DeWitt, PhD, Special Projects Officer – RFA 13-07

November 5, 2013

# Webinar Objectives



- Overview CIRM Alpha Stem Cell Clinics (CASC) Network:
  - Goals
  - Organizational Structure
- Description of RFA 13-06 Alpha Clinics Award
- Description of RFA 13-07 Coordinating and Information Management (CIMC) Award



# Overview of the CASC Network Mission

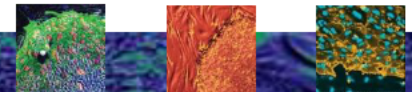
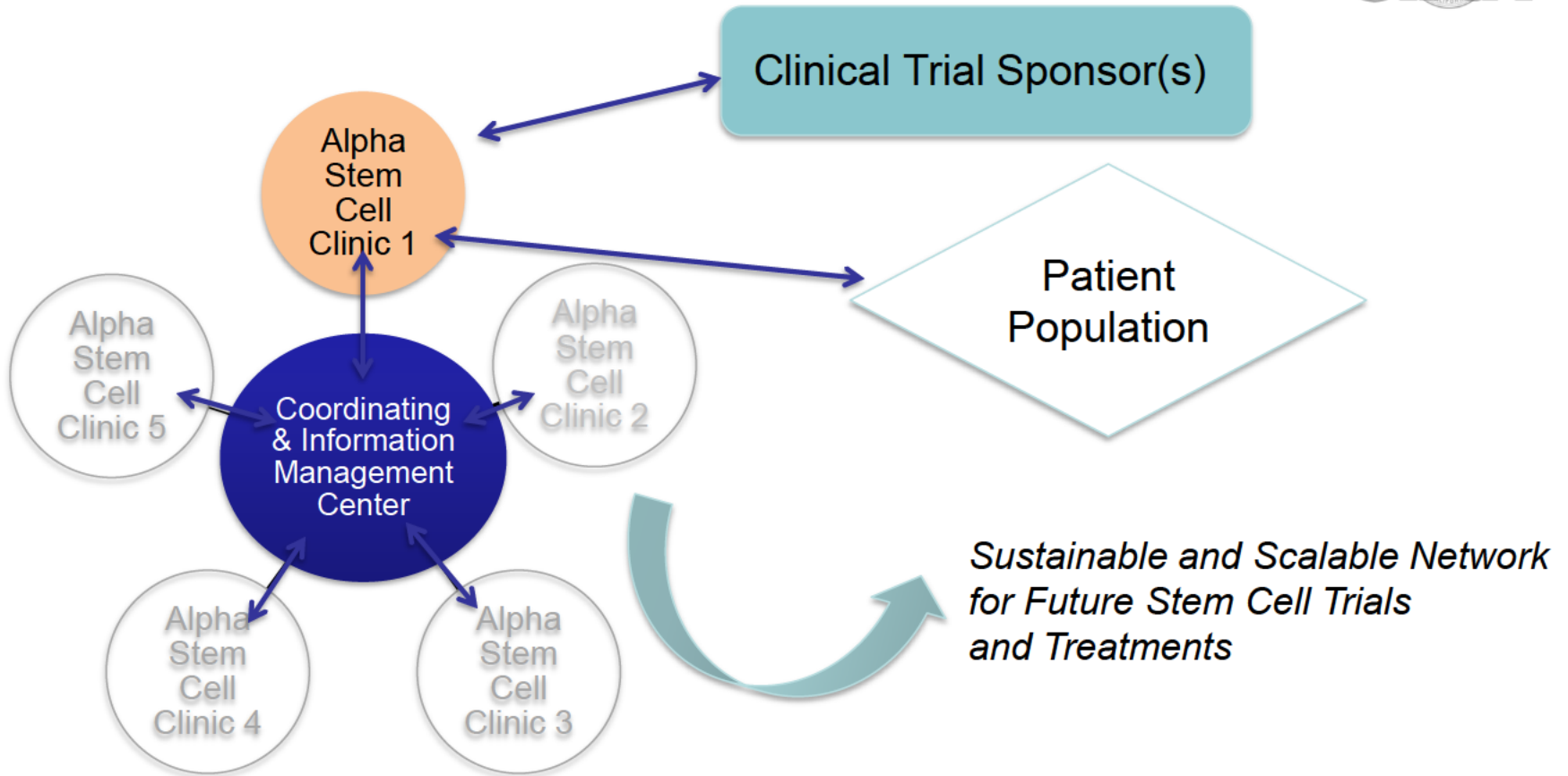


Create an efficient, high quality, sustainable infrastructure to accelerate stem cell therapy development:

- Support stem cell trials and approved treatments
- High quality data and information to inform the field
- Educate patients and the public
- Facilitate healthcare economics models for stem cell treatments



# CASC Organization and Interactions



# CASC Steering Committee will Coordinate Network Activities



## Composition:

Alpha Stem Cell Clinics Program Directors (PDs), CIMC PD, CIRM representative, *ad hoc* expert advisors

## Activities:

- Establish guidelines to promote data and knowledge sharing for the Network
- Develop quality standards
- Create efficiencies and shared resources for the CASC Network
- Support the overall mission of the CASC (e.g. address challenges and provide advice/assistance for operational and strategic issues for the CIMC, Alpha Stem Cell Clinics and OET program)



# RFAs for the CASC Initiative

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RFA13-06: Alpha Stem Cell Clinic Sites: Up to five clinical sites located in or affiliated with existing academic centers (up to \$55M over five years for all sites)

RFA13-07: One Coordinating and Information Management Center (up to \$15M over 5 years)

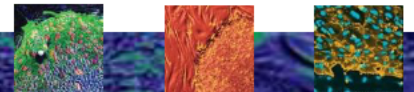


# Objective of RFA 13-06 CASC- Alpha Clinics Award

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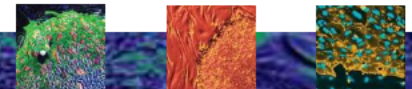
The Alpha Stem Cell Clinics will each reside within a California Academic Medical Center to provide critical operational support for the conduct of clinical trials for investigational stem cell therapies and to operate as a center of excellence for approved stem cell therapies.



# CASC – Alpha Clinic Activities



- **Operational Support for Clinical Trials**
  - Patient screening, enrollment, clinical trial monitoring support, patient coordination and scheduling; coordinate with services, standards provided by CIMC
- **Data and Information Management**
  - Assist Sponsor with data and information management and integration of appropriate elements into CIMC shared databases with appropriate protections
- **Outreach Education and Training**
  - Interact with CIMC OET program and counselors to provide patients with information and education related to stem cell clinical trials
- **Healthcare Economics**
  - Assist with the outward facing interactions of the CASC with regards to patient care coverage and reimbursement issues. i.e. utilizing hospital resources, assist patients in accessing appropriate access to their healthcare coverage. Contribute to CASC evidence base to support the development of sustainable business models including reimbursement strategies





# Out of Scope Activities Not Supported by RFA 13-06



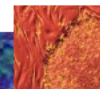
- Construction
- Pre-clinical research and IND-enabling studies.
- Process development.
- Cell manufacturing.
- Clinical trial costs (*Clinical Trials come in with independent funding*).



# Institutional Eligibility

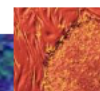
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- One Application per Institution
- Accredited Medical Center in California
- Academic Medical Center (or affiliate)
- Non-profit or For-Profit
- *Applicant Institutions for RFA 13-06 can also submit an LOI for RFA13-07 CIMC but with different PDs*



# Program Director Eligibility

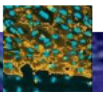
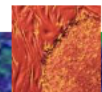
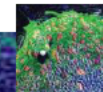
- Nominated by the Institution's Authorized Executive Officer to be the sole applicant from that Institution
- Independent Investigator
- MD or equivalent with an active California medical license and hospital privileges at the applicant institution's medical center
- Minimum of 30% Effort



# Project Eligibility- Lead Clinical Trials



- A minimum of two Lead Clinical Trials.
- The investigational product(s) within scope of this RFA i.e. stem/progenitor cell therapeutic product \*Note: “minor” modifications to current cell therapies are out of scope
- An IND submitted for at least one of the Lead Clinical Trials at the time of application submission.
- Committed sponsor and source of independent funding for the proposed Lead Clinical Trials
- Clinical trial agreements and/or letters of intent to enter a clinical trial agreement with the clinical trial sponsors of the lead clinical trials.
- At least one clinical trial initiated within 12 months of the award date (or in progress).



# Review Criteria

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- (1) Responsiveness to the RFA
- (2) Institutional Support and Sustainability Plan
- (3) Alpha Stem Cell Clinic Team
- (4) Implementation and Operational Plan
- (5) Alpha Stem Cell Clinic Lead Clinical Trials



# Review Criteria- Examples of Considerations

**Institutional Assets:** existing infrastructure, systems (i.e shared IRB review), track record with stem cell clinical trials

**Team experience, track record, strength of organizational plan:** Experience in managing and supporting regulated clinical trials especially in cell therapy. The plan and participation for the overall mission of the CASC (i.e. “shared knowledge” and centralized efficiencies)

**Lead Clinical Trials:** Note: RFA priorities for proof of concept end points & California sponsor; rigor of evaluation process for funding; “fit” between proposed Alpha Clinic and trials

**Feasibility and Appropriateness of Timeline and Budget**

**Commitment** to and likelihood of **sustaining a stem cell focused** clinic beyond the initial funding period



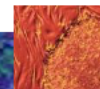
# Prepare early for Application



- Pay close attention to the eligibility criteria and need to provide supporting documentation
- Only one application per Institution requiring AEO signature

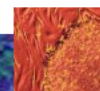
*AEO (e.g. Vice-Chancellor, Dean, etc.) has the authority, or delegated authority, to nominate the institution's sole applicant for RFA 13-06 and to commit the Institution's resources to support the Alpha Clinic activities*

- Assemble team and lead clinical trials (Sponsors and Funders)



# Key Information for Application

- Institution and team's track record for regulated trials
  - Highlight stem cell or cell therapy trials experience where present
- Description of the nature of evaluation and funding of lead clinical trials
  - Due diligence reports for corporate or private sponsors, description of review process for grant funding, etc.
- Clinical protocol synopsis, target product profile, trial budget (confirming independent funding), manufacturing plan summary
- FDA correspondence related to lead clinical trials
  - Unresolved or FDA "hold" issues must be indicated with a detailed plan and timeline to address these issues



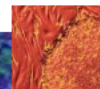


# Key Information for Application

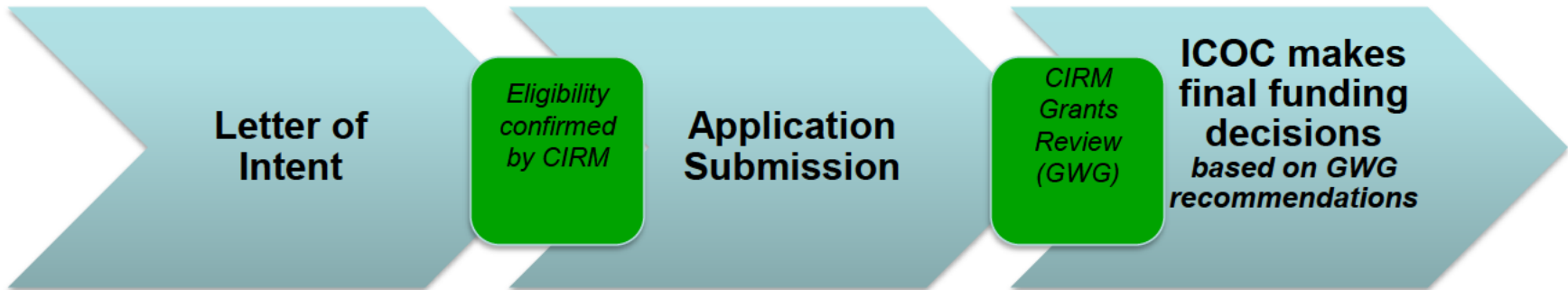
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- Timeline and milestones should be clear
- Budgets must be well justified – activity based budget aligned with operational and implementation plan
- Letters of support: sponsors, funders, future projects



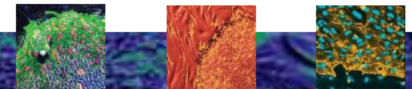
# Application and Funding Process for RFA 13-06



- AEO signature
- Applicant Certifications re. scope and eligibility
- Agreement to participate in Steering committee, if awarded
- Identify Site and Team
- Description of two Lead Clinical Trials
- IND # for at least 1 Lead Clinical Trial

- Application Form with Public Abstract
- Proposal (Alpha Clinic and Lead Clinical Trials)
- Documents, i.e. clinical trial sponsors/funding source, regulatory status, hospital accreditation, supporting letters
- Activity-Based Budget
- Biographical Sketches

- Pre-Funding Administrative Review
- Notice of Grant Award with Milestones & Terms
- Quarterly Progress Reports

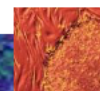


# CIRM's ICOC has allocated up to \$55 million to fund up to 5 Alpha Stem Cell Clinics



## Key dates to remember:

Letter of Intent Due	December 16, 2013
CIRM Eligibility Notification	January 13, 2014
Application Due	March 14, 2014
Grants Working Group review	Spring 2014
ICOC Consideration	Summer 2014
Earliest Funding	Fall 2014

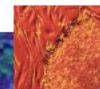


# RFAs for the CASC Initiative

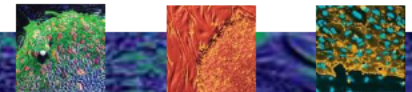
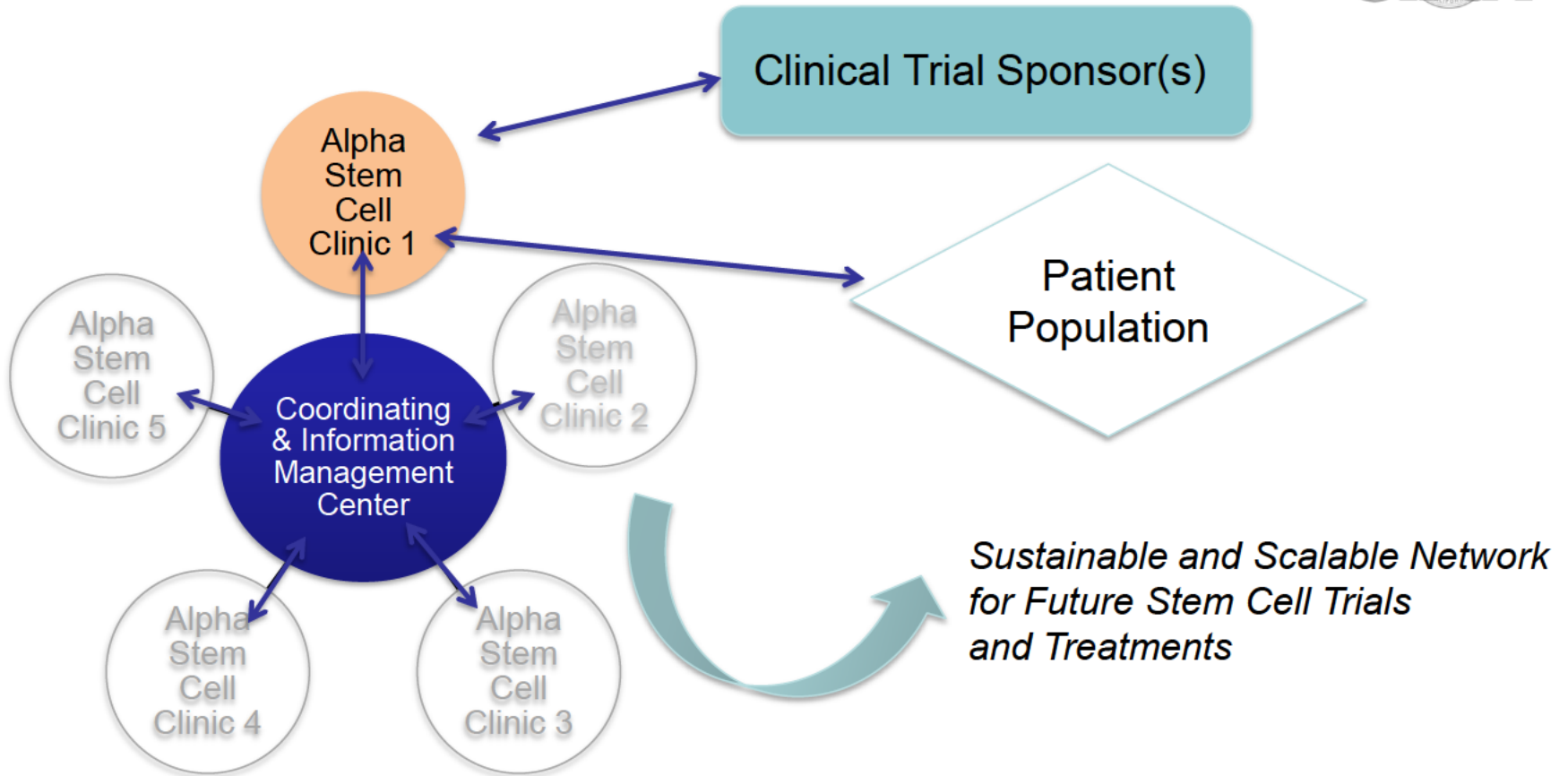
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**RFA13-06: Alpha Stem Cell Clinic Sites: Up to five clinical sites located in or affiliated with existing academic centers (up to \$55M over 5 years for up to 5 sites)**

**RFA13-07: One Coordinating and Information Management Center (CIMC up to \$15M over 5 years)**



# CASC Organization and Interactions



# Objectives of RFA 13-07; CASC – Coordinating and Information Management Center



To provide critical input and support to the Alpha Stem Cell Clinics sites on clinical trial design and operations, data and information management, public and patient education and develop healthcare economics resources to inform the field



# CASC - CIMC Activities



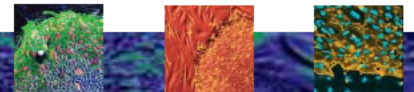
- Clinical trial support
- Data and information management
- Outreach, education and training
- Development of healthcare economics resources



# CASC - CIMC Activities



- Assist Alpha Stem Cell Clinics with clinical trials and delivery of approved therapies
  - e.g. Clinical operations, monitoring, regulatory, efficiencies (master agreements, model contracts, templates, centralized IRB resources)
- Data and information management
  - e.g. Clinical data, biostatistical analysis and support, assistance with FDA/DSMB/investor presentations, knowledge sharing resources

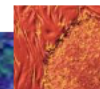




# CASC - CIMC Activities



- Outreach Education and Training (OET)
  - Create tools and provide information to inform public and patients on stem cell therapies in clinical trials
  - Work with CIRM and Alpha Clinic Sites on education and use of patient counselors as part of OET program
- Healthcare Economics
  - Use expertise, data, and knowledge to inform clinical trial decisions
  - Develop an evidence base to inform development of sustainable business models including reimbursement strategies



# Financial Structure for CIMC Activities

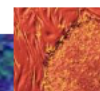
- Propose “minimum” slate of services on non-fee basis (supported by grant)
  - To provide clinical support for lead clinical trials from Alpha Clinics
  - Establish OET and Healthcare economics programs
- Propose additional services, at reasonable but competitive fees, for non-slate services and clinical trial activities



# Organization Eligibility



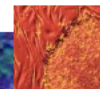
- One Application per organization
- Experience:
  - A minimum of 5 years continuous experience in providing clinical trials and data management services (LOI Track Record Template)
- Assets:
  - Existing infrastructure to support clinical operations and data management
  - Financial stability: “cash flow-neutral” over one year prior to application (documentation must be provided in LOI)
- Location: At time of application must be
  - California-based or provide letter of intent to enter into a purchase or lease agreement for a CA location
  - Must be in CA before issuance of NGA and be able to initiate activities within 3 months of grant start date as indicated on NGA



# Program Director Eligibility



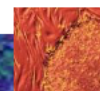
- Employed at applicant organization
- PD is the Organizational Official or is directly reporting to one
- Minimum 30% effort to the CASC - CIMC
- Extensive experience in
  - leadership and management
  - clinical trial services and information and data management (see LOI Template)
- Letter of commitment from applicant organization for
  - Staffing
  - Infrastructure
  - California location



# Review Criteria

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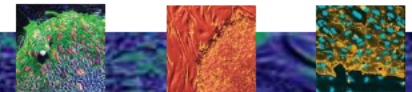
- (1) Organizational strength, assets, commitment and sustainability plan
- (2) Qualifications and track record of the applicant PD and team
- (3) Implementation plan
- (4) Responsiveness to the RFA
- (5) Appropriateness of the budget.



# Review Criteria – Examples of Considerations

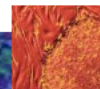


- **Timeline and Feasibility to initiate operations in California**
- **Applicant organization-** highlight key aspects of the track record and indicate how resources would be leveraged for the CASC
- **Appropriateness of Budget-**strength of the proposed “minimal slate” of services for Alpha Stem Cell Clinics that would be covered by the Budget under this RFA
- **Financial Strength and Sustainability plan-** degree to which this organization would create a scalable sustainable operations to support the CASC Network beyond the 5-year funding period



# Review Criteria – Depth and Quality of Experience

- **Staffing proposal-** highlight the experience of proposed staff with
  - Clinical trials
  - Data/information management- confidentiality, tiered access, data analysis
  - OET and Healthcare Economics- describe experience and assets that can be leveraged



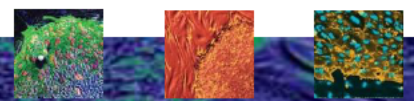
# Application and Funding Process for RFA 13-07



- *Applicant Certification of Eligibility*
- *Track Record Template*
- *Financial Information (demonstrating at minimum cash flow neutral over past year)*

- *Proposal*
- *Supporting Documents*
- *Expanded Track Record Template*
- *Additional Financial Information*

- *Pre-Funding Administrative Review*
- *Notice of Grant Award with Milestones & Terms*
- *Quarterly Progress Reports*





# CIRM's ICOC has allocated up to \$15 million to fund a Coordinating and Information Management Center



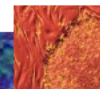
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Earliest Funding	Fall 2014



# A Few Tips for Success

- **Know your audience** – who are you trying to convince with your proposal – it's the reviewers with product development, disease, clinical, preclinical, manufacturing expertise and experience, and it's CIRM – know CIRM's mission and read the RFA to understand what's needed
- **Ask questions as you prepare the application** – don't guess, ask CIRM if you are unsure. All potential applicants should pick up the phone or email CIRM – let us know you are interested and let us try to help you
- **Reserve time to write the application** – a competitive application requires focus



# Contacts at CIRM:

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## For information about RFA 13-07:

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## For information about the review process:

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