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The state stem cell agency

Strategic Partnership I Funding Initiative: RFA 12-05

Educational Webinar for Potential Applicants April 25, 2012

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Providing tips to help you better prepare your application – our Webinar objective

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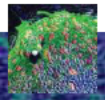
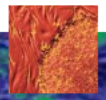
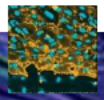
- We will review the following:
  - Goals and intent of the RFA
  - Tips for Success
  - Context of this initiative within CIRM's portfolio
  - Eligibility criteria e.g. of therapeutic candidate, Principal investigator, and Organization
  - Review Criteria for this award
  - Templates to guide your organization of the material
  - Contact Info – how to reach us at CIRM
- We're here to answer your questions and help better position you for success

RFA's goals and intent – attract industry in completing a clinical trial

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- Purpose is to **attract industry engagement and investment in CIRM funded stem cell research**.
- **Objective** of the first call under this Initiative, the Strategic Partnership I Awards, **is to achieve, in 4 years or less, the completion of a clinical trial** under an Investigational New Drug (IND) application filed with the Food and Drug Administration (FDA).
  - Completion of a phase I, phase I/II or phase II clinical trial

Tips for Success – an aid, not a promise!

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- **Start with a great idea** – we want your best ideas and research to move forward, and a strong rationale. If this program isn't of top interest for your company, you're unlikely to convince the reviewers it should be CIRM's
- **Explain how this program fits into your overall company strategy**, as well as other compounds/programs for the same target/indication
- **Preliminary data** is important – provide it!
- **Show the data**, not just your interpretation of the data – reviewers base their recommendations on the evidence, not on your hopes

Tips for Success – an aid, not a promise!

- **Describe** what and where the **risks** are, **and plans to mitigate or remove them** – it shows you are on top of the issues and are thinking of all sides; painting a rosy picture and not addressing challenges or risks weakens your application
- Address novelty vs probability of success and disease impact
- **Address commercial viability** of your proposed therapy
  - Differentiate from potentially competing therapies and current standard of care
- **Address freedom to operate** and any investment you've made to ensure it

Tips for Success – an aid, not a promise!

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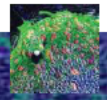
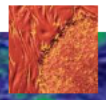
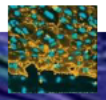
- **Milestones should be clear and important** - what are the critical experiments for that Go-No Go decision to ensure you have the "truth" about whether or not to proceed
- B**udgets should be well justified -** be good stewards of the $ and provide the rationale for what you really need to conduct the studies and answer the key questions or issues
- **Timelines should be well reasoned** – provide rational, realistic time frames

Tips for Success – read the RFA!

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- **Read the RFA** – make sure you understand what's being asked and be sure to address the points
  - As examples: Show the aspirational Target Product Profile; the development plan; studies to provide the evidence; knowledge of the regulatory steps; data to show your therapeutic candidate is ready to do the IND enabling steps; documentation that you have had a discussion with the FDA about your product and plans; documentation that you have the legal ability to move your product forward to patients; provide a clinical trial protocol that is well-designed, has safety parameters in place for patients, and is designed to answer the key questions you need to make a decision about whether to move the product forward into later stage development

Tips for Success – an aid, not a promise!

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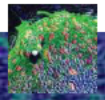
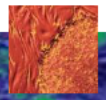
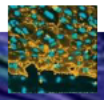
- **Know your audience** – who are you trying to convince with your proposal – it's the reviewers with product development, disease, clinical, preclinical, manufacturing expertise and experience, and it's CIRM – know CIRM's mission and read the RFA to understand what's needed
- **Ask questions as you 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

Examples of what NOT to do

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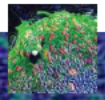
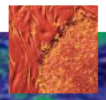
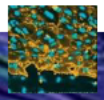
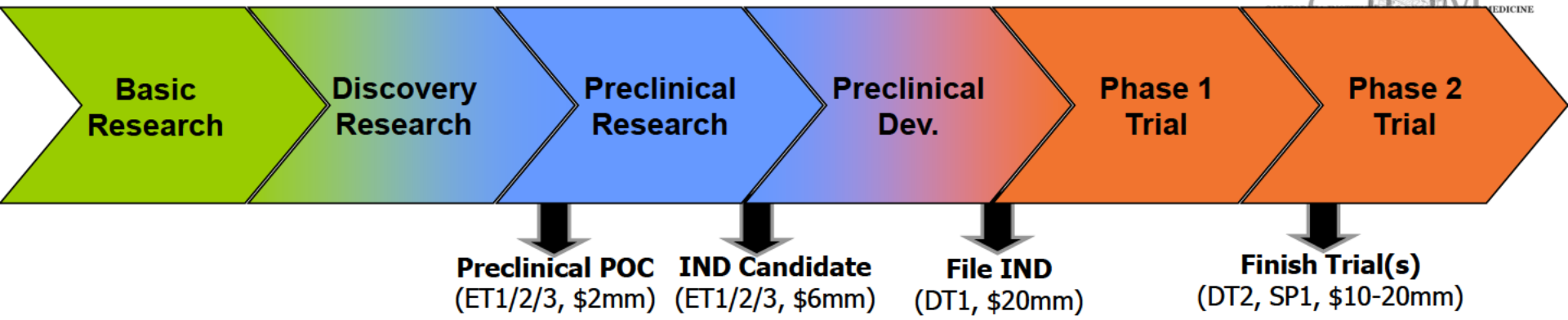
- Propose a large, multi-center, multi-national trial without any evidence you have tested your proposed product in any human
- Propose a clinical indication with no or weak rationale, that is peripheral to the company's main priorities that have much stronger scientific and clinical rationale
- Dismiss or ignore data that doesn't fit with your rationale – go over the data and explain it, state the studies you will do that can answer the question
- List completed studies without providing the data – drafts are informative and it's important to be transparent about your data
- Name a principal investigator with no or very limited product development experience in conducting or successfully guiding studies into the clinic

Examples of what NOT to do

- Provide the idealized view, with no or minimal information on potential scientific, technical, regulatory, or clinical risks or challenges to the project and how you will address them
- Request $millions for “toxicity studies” and provide a one sentence rationale and description of what you intend to do with the $ - all activities, particularly expensive ones, require appropriate justification
- Take the published award ceiling amount and divide by the number of years to arrive at your annual budget needs
- Propose budget and cost sharing for expenses/activities outside the scope of the CIRM funded project
- Propose an unrealistically optimistic time frame – could impact on reviewers’ perception of team’s experience

Where does this initiative fit in CIRM's current translational portfolio



Early Translational I,II (ET1, ET2)

Disease Team I (DT1)

42 current translational programs

Early Translational III (ET3)

Disease Team II (DT2)

Strategic Partner I (SP1)

Scope criteria - must be single therapeutic candidate

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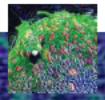
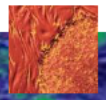
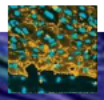
- Cell therapy candidate
  - derived from/utilizing pluripotent stem cells, progenitor/adult tissue-derived stem cells; or adult stem cells modified either pharmacologically or genetically (where the modification is for the purpose of correcting a disease phenotype or is critical to achieve the therapeutic strategy)
- Small molecule or biologic candidate
  - mobilizes endogenous stem cells to promote tissue repair/regeneration and for which there is convincing evidence of such activity; or identified and characterized using patient-derived induced pluripotent stem cells or their derivatives; or is specifically targeted to destroy cancer stem cells, based on serial clonal transplantation assays in an in vivo model

What's out of scope for this first round

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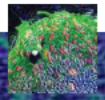
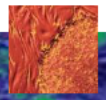
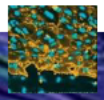
Outside of Scope and Specifically Excluded:

- minimally manipulated bone marrow cells; umbilical cord blood stem cells; adipose-derived stem cells; and unmodified hematopoietic stem cells

Eligibility criteria for PI and organizations

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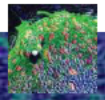
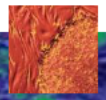
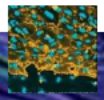
- PIs must have an MD, PhD or equivalent degree and authorized by applicant organization to conduct the proposed research in California
  - PI is an employee of applicant organization with demonstrated expertise in product development and in managing clinical research programs
  - Documented authority from applicant organization to staff the proposed project in California
  - Documented commitment from applicant organization to provide resources sufficient to carry out research

Applicants must show evidence of commercial validation

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- Show evidence of either having the financial capacity to move the project through development or of being able to attract the capital to do so. This may be evidenced by, for example:
  - significant investment by venture capital firms, large biotechnology or pharmaceutical companies and/or disease foundations; or a licensing and development agreement with a large biotechnology or pharmaceutical company, or a commitment to enter into such an agreement executed prior to the disbursement of CIRM funding.

Reviewer criteria to assess the application

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- Applications will be evaluated by Grants Review Group in five key areas:
  - Significance and impact
  - Risk/Benefit
  - Design and feasibility
  - Principal investigator, development team and leadership plan
  - Quality of collaborations, assets, resources and environment
- Intellectual Property and Industry subcommittee, ICOC will evaluate applications in one key area:
  - Evidence of commercial validation

Templates to guide the organization of your submission

- CIRM Target Product Profile (TPP)
- CIRM Clinical Protocol Synopsis
- CIRM Manufacturing Plan Synopsis
- Other key information you will need to provide includes:
  - Due Diligence Report
  - FDA correspondence
  - Clinical Protocol and Investigator Brochure
  - Copies of authorization for cross reference of Drug, Device or Facility master files
  - Licenses and agreements (MTAs)
  - Evidence of Commercial Validation
  - Related Business Entities Disclosure Form (Adobe PDF template)

CIRM's ICOC has allocated up to $30 million for 3 awards: Key dates to remember

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Letter of Intent due	May 16, 2012
Award Applications due	June 26, 2012
Grants Review Group review	September 2012
Intellectual Property and Industry Subcommittee, ICOC assess evidence of commercial validation	Quarter 4 2012
ICOC Consideration	Quarter 4 2012
Earliest Funding	January 2013

Contact us if you have any questions

- **For information about this RFA:**
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