CALIFORNIA'S STEM CELL AGENCY

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Accelerated Development Pathway: PA 14-01

Educational Webinar for Potential Applicants March 19, 2014

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Webinar objective: Providing tips to help you better prepare your application

- We will review the following:
  - Overview of Program Announcement 14-01
  - Goals and Intent
  - Award Information and Scope
  - Review Criteria
  - Application Requirements
  - Tips for Success
  - Key Dates
  - Contact Info – how to reach us at CIRM
- We're here to answer your questions and help better position you for success

PA 14-01 overview: The Accelerated Development Pathway

- **Purpose** of the Accelerated Development Pathway is to advance the progress of clinical projects in the CIRM Disease Team and Strategic Partnership portfolio.

- **Open to** current grantees who hold a Disease Team or Strategic Partnership Award* (the “Parent Award”) that includes funding for a clinical trial.

- CIRM intends to offer **future opportunities** for application into the Accelerated Development Pathway.

* Includes: RFA 09-01, RFA10-05, RFA 12-05, RFA 12-09, RFA 13-01

PA 14-01 overview: The Accelerated Development Pathway

- Applicants who are accepted into the Accelerated Development Pathway will have:
  - Continued support toward achieving milestones on the Parent Award.
  - Additional access to CIRM Scientific Staff and External Advisors.
  - Access to expedited funding for recommended key development activities (that does not depend on subsequent RFA cycles or require re-application).

- Applicants who are not accepted into the Accelerated Development Pathway at this time will have:
  - Continued support toward achieving milestones on the Parent Award.

PA 14-01 goals and intent: Accelerate progress to clinical safety and proof of concept

- Support CIRM's mission to bring stem cell therapies to the people of California.
- Accelerate development of high impact stem cell-based therapeutics currently in the CIRM clinical portfolio.
- Identify teams who are making rapid progress on their Parent Award and provide them with additional resources to further accelerate their advancement toward demonstration of an acceptable safety profile and clinical proof of concept for the stem cell-based therapy under development.

PA 14-01 process: Application into the Accelerated Development Pathway

- Applicants propose additional activities to accelerate progress with the therapeutic candidate in the therapeutic area funded under their Parent Award.

- Grants Working Group (GWG) reviewers will review each proposed activity and its associated budget, in a modular manner, according to the Review Criteria in PA 14-01.

- GWG reviewers will recommend:
  - Whether an applicant team should be included in the Accelerated Development Pathway program at this time.
  - For recommended applicants, which accelerating activities proposed by the team should be considered for funding.

- CIRM's ICOC will consider recommendations of the GWG

PA 14-01: Eligibility criteria

- Current grantees who hold a Disease Team or Strategic Partnership Award* (the "Parent Award") that includes funding for a clinical trial.
- Applicant organization the same as on the Parent Award
- For profit and non-profit organizations are eligible
- PI and co-PI (if applicable)
  - Expected to be the same as on the Parent Award
  - Expected to maintain at least the percent effort committed in the Parent Award for accelerating activities under PA 14-01

* Includes: RFA 09-01, RFA10-05, RFA 12-05, RFA 12-09, RFA 13-01

PA 14-01 award: Funding available through the Accelerated Development Pathway

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- CIRM's ICOC has committed up to $200 million to accelerate progress of early clinical projects in the CIRM Disease Team and Strategic Partnership portfolio

- To support future grantees and current grantees whose projects are not yet mature enough for the Accelerated Development Pathway, CIRM intends to offer **future opportunities** for application into the Accelerated Development Pathway.

PA 14-01 award: Funding available through the Accelerated Development Pathway

- Activity-based funding
  - **A maximum per project team of $25 million in total cost funding**
  - Up to a maximum of $5 million for manufacturing improvements
  - Up to a maximum of $10 million for key development activities
  - Up to a maximum of $20 million to conduct a follow-on clinical trial
- Each activity proposed to accelerate program progress must be supported by a well-justified, modular budget
- May include direct project costs, direct facilities costs, and indirect costs

PA 14-01: Award mechanism

- Awards made under PA 14-01 for activities that support the clinical trial funded under the Parent Award will be funded under a supplement to the Parent Award and will use the same mechanism as the Parent Award (loan or grant)

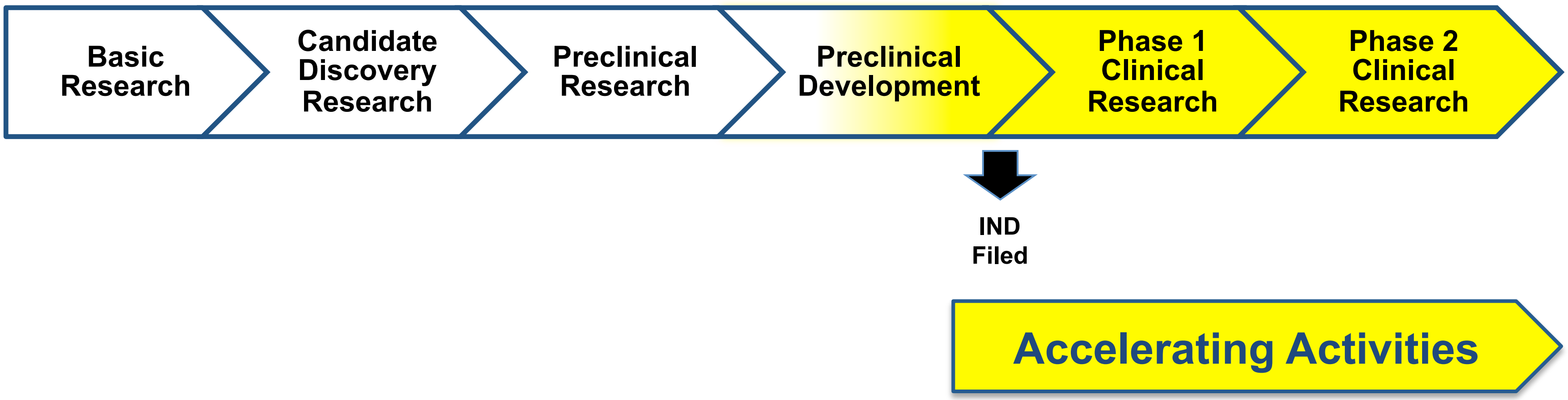
- Funding for a follow-on clinical trial will be in the form of a new award and may be in the form of a loan or grant

PA 14-01: Co-funding requirements

- If Parent Award includes a co-funding requirement:
  - A supplement to the Parent Award will carry the same co-funding ("matching") requirement as the Parent Award
  - Confirmation of matching funding is not required until activation of the supplemental funding under PA 14-01
  - A new award for a follow-on clinical trial will require matching funds equal to at least 1/3 of the costs of the new award (may include in-kind support)
- If Parent Award does not have a co-funding requirement:
  - PA 14-01 does not introduce new requirements for matching funding to support approved accelerating activities

PA 14-01 scope: Activities to accelerate clinical development of stem cell therapeutic

The Accelerated Development Pathway is designed to support ongoing, ***mature*** programs that are in, or near, **Early Clinical Development**



- May include manufacturing, nonclinical and clinical activities
- May include add-ons to the clinical trial funded in the Parent Award
- May include a follow-on clinical trial to demonstrate proof of concept

PA 14-01: In-scope accelerating activities

- Accelerating activities are intended to support the same therapeutic candidate in the same therapeutic area that is funded under the Parent Award
- Manufacturing activities
  - Process or device optimization, assay validation, scale-up
- Nonclinical activities
  - Biomarker validation, bridging studies, assay development
- Clinical activities to augment the supported clinical trial
  - New trial sites, additional outcome assays, expanded patient group
- Follow-on clinical trial to demonstrate proof of concept

PA 14-01: Out of scope activities

- Phase 3 clinical studies
- cGMP manufacturing to support Phase 3 clinical studies
- Clinical trials using second generation therapeutic candidates
- Clinical trials testing the therapeutic candidate in a new clinical indication
- Non-interventional clinical studies or clinical studies that do not include administration of the therapeutic candidate
- Development of a medical device not required for administration of the therapeutic candidate
- Development activities that are the subject of a funding obligation by a third party

PA 14-01: Review criteria

- Applications will be evaluated by the GWG for scientific and technical merit, according to the following criteria:
  - Clinical competitiveness and impact of the proposed therapy
  - Relevance of the therapeutic to regenerative medicine
  - Strength of the development program
  - Proposed activities to accelerate the development program
  - Feasibility of the proposed activities to accelerate development
  - Qualifications of the development team
  - Progress on the Parent Award and effective program leadership

PA 14-01: Application forms

- Part A: Application information form
- Part B: Proposal for accelerated development plan
- Part C: Biographical sketches for key personnel
- Part D: Activities-based budget for proposed accelerating activities
- Part E: FDA correspondence
- Part F: Updated clinical protocol for trial funded in Parent Award
- Part G: Updated investigator brochure for trial funded in Parent Award
- Part H: Clinical protocol for follow-on clinical trial (if applicable)
- Part I: Investigator brochure for follow-on clinical trial (if applicable)
- Part J: Updated intellectual property and licensing agreements

- CIRM will provide to the GWG the most recent progress report from Parent Award and briefing document prepared for CDAP interaction

PA 14-01 application: Proposal to accelerate your progress and clinical development

- Update TPP, clinical protocol and investigator brochure
- Provide a summary of current progress on the Parent Award and provide a timeline for remaining milestones
- Discuss competitiveness and impact of your therapy in relation to the current clinical landscape
- Propose new development activities that, if conducted concurrently, would increase efficiencies and inform decisions that could facilitate demonstration of clinical safety and proof of concept
- Indicate how each new activity will augment and accelerate clinical development of the stem cell therapeutic funded in the Parent Award
- Present each new activity in a modular manner to include the requested funding it would require and the timeframe across which it would be conducted

PA 14-01: Tips for success

- Know your audience
  - GWG: reviewers with product development, disease, clinical, preclinical, manufacturing expertise and experience
  - CIRM: know CIRM's mission and read the PA to understand what's needed
- Ask questions as you prepare the application
- Reserve time to write the application
- Produce budgets that are well reasoned and reflect a clear rationale for necessary expenditures
- Provide rational, realistic time lines that incorporate mitigation strategies
- Propose milestones that are clear and meaningful

PA 14-01: Examples of what NOT to do

- Take the maximum funding amounts published for each type of activity and divide by the number of proposed activities to arrive at your supplemental budget request
- Propose budget and cost sharing for expenses/activities outside the scope of activities to accelerate the CIRM funded project
- Propose an unrealistically optimistic time frame
- Propose activities with weak rationale that would not facilitate progress toward demonstration of clinical proof of concept
- Provide an idealized view that does not reflect potential risks or challenges to the project and how you will address them

PA 14-01: Key dates to remember

Letter of Intent Due	March 24, 2014
Application Due	April 28, 2014
Supplemental Information Due	June 5, 2014
Grants Working Group Review	July 14-15, 2014
ICOC Consideration	Fall, 2014

PA 14-01: Contact us if you have any questions

- For information about PA 14-01:

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- For information about the review process:

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