CALIFORNIA'S STEM CELL AGENCY

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Prioritization – issues to consider

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Prioritization – what is it?

- What is prioritization, in the context of the Scientific Advisory Board recommendations?

Identify 6 to 8 development projects (from currently funded disease teams or strategic partnerships, while still allowing a porous window for new mature projects to competitively enter) that have the potential to reach clinical proof of concept in/by 2017, consistent with CIRM's Strategic Plan, and facilitate their movement through CIRM's "Accelerated Pathway"

Accelerated Pathway – what is it?

- Accelerated pathway
  - More frequent and extensive discussions with Clinical Development Advisors and CIRM scientific staff on preclinical, manufacturing, regulatory, clinical, and commercial aspects of developing the therapy
  - Finance essential development components as they arise, and as needed, follow-on phase 2 trials.
  - As projects will be selected from already GWG recommended and ICOC funded solicitations (disease teams and strategic partnerships), will already have CIRM funding for early phase clinical trials

Prioritization

- Seek your decisions, input on two main issues
  - Process
  - Criteria for review

Clinical Proof of Concept – value to patients, the public, and to investors

Scientific Advisory Board recommendation directly aligns with clinical goal of CIRM's Strategic Plan, to advance stem cell science towards evidence of safety and activity in patients e.g., clinical proof of concept

Clinical Proof Of Concept

- Tangible endpoint that has meaning for patients and the public who brought CIRM into existence
- Important inflection point for attracting investors and moving towards commercialization

Options to consider for process – what, how, who and when

- What projects to review?
  - Currently funded (by Q1 2014) projects with potential for reaching clinical proof of concept in/by 2017 – Disease Teams and Strategic Partnerships

Options to consider for process – what, how, who and when

- How to review? Who will review?
  - Projects brought to GWG to review and recommend 6 to 8 for Accelerated Pathway to ICOC, including budgets and assessments for follow-on phase 2, where required and appropriate, if milestones are met

Options to consider for process – what, how, who and when

- ICOC approved "Accelerated Pathway Projects" will receive increased access to external and internal expertise from Clinical Development Advisors and CIRM scientific officers AND CIRM funds for essential development components and milestone-driven projects through clinical proof of concept (within development funds, not dipping into other funding categories)

- When?
  - Timeline for completion of prioritization and presentation to ICOC 1st half of 2014

Options to consider for criteria – what does CIRM really want? Make it clear

- Stem cell therapies where the stem cell connection is strong and compelling
- Clear and strong plan for the development pathway
- Potential for major impact is strong
- Diseases with an accepted or reasonable marker of activity relevant to the disease, some (preferably good) understanding of the mechanism and pathophysiology of the disease, and what it takes to establish efficacy, such that clinical trials with well-defined, biologically quantifiable endpoints can be planned, and primary clinical endpoint is clear and well established
- Proof of concept possible in/by 2017
- Strong, credible team with expertise in development and ability to execute on plans

Budget

- $200M for follow-on phase 2 trials, where required and appropriate to achieve clinical proof of concept, for up to 6 to 8 projects
  - Funding would be setaside from already designated funds for Development – see funding plan attachment 6-B1