CALIFORNIA'S STEM CELL AGENCY

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Basic and Translational Research ICOC meeting Agenda Item 6C6

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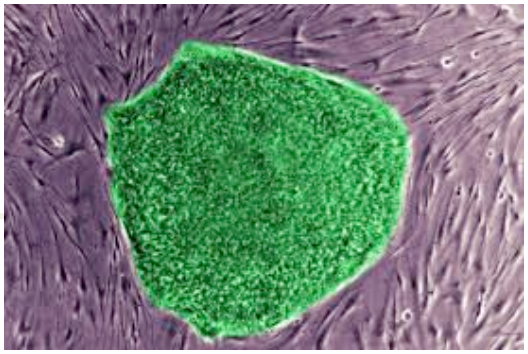
CIRM Basic Biology Awards



- Program Objectives
  - support studies leading to an understanding of fundamental cellular and molecular mechanisms underlying stem cell behavior
  - provide new insights into disease mechanisms
  - focus on human pluripotent and progenitor cells
- Research Themes
  - stem cell pluripotency
  - cell differentiation mechanisms
  - stem cell genomic stability and heterogeneity
  - disease and tissue models derived from stem cells
  - cellular reprogramming
- Status
  - 4 rounds funded, 83 projects awarded for $115 MM; a fifth round is pending ICOC decision in January

Basic Research

David Botstein on the value of basic research

- “I start with the premise that we understand very little of the world. Specifically, we understand a tiny fraction of what’s written in our genomes. We understand a tiny fraction of what parts of medicine work well, and what parts are just tradition. ….The value of basic science, of course, is once we do understand something we might be able to do something.”

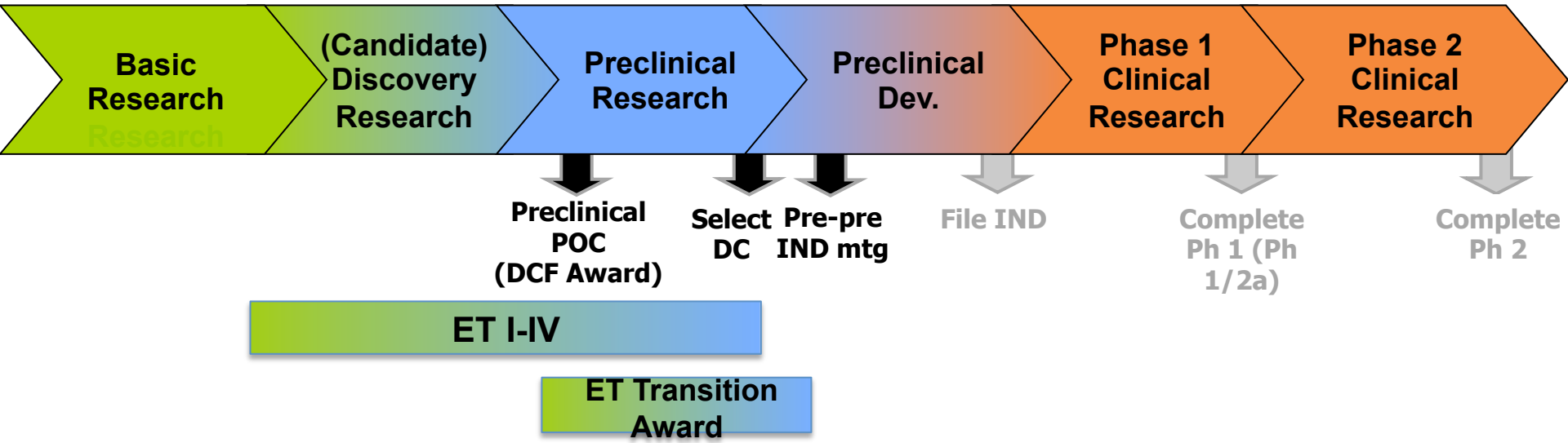
Basic Research

- SAB/CIRM management recommendation: continue support for basic research
- 2012 funding plan for Basic Research
  - Scenario 1: $100 MM for 3 more Basic Biology rounds ($35 MM, $35 MM, $30 MM)
  - Scenario 2: $70 MM for 2 more Basic Biology rounds ($35 MM, $35 MM)
- Proposed funding plan for Basic Research
  - 3 more Basic Biology rounds, ~ $30 MM per round

Translational Research

- 2012 funding plan for Translational Research
  - Scenario 1: 1 more round (ET V) at $65 MM; 1 more small translational award (e.g. Tools and Technologies IV at $30 MM)
  - Scenario 2: 1 more round (ET V) at $60 MM
- Translational Research 'Unallocated' – $44 MM
- Proposed funding plan
  - For promising existing Early Translational programs
    - Reallocate funds ($69-99 MM) to Development to contribute to IND enabling/clinical development for ET projects (along with existing Development funds and potential public/private funding)
    - Fund a Transitional Award Program to move existing promising DCF awards to Development candidates/up to pre-pre IND meeting ($40 MM for 2 rounds for awards of ≤ 2 years)

Early Translational Program



ET (I-IV) Program: Within 3 years,

•Achieve *in vitro* or *in vivo* proof of concept, or

•Achieve a development candidate (DC) ready to move into IND-enabling preclinical development

Proposed ET Transition Award: Within ≤ 2 years,

- Preclinical POC (DCF Award) -> DC or Hold pre-pre IND meeting with FDA
  - Rationale: Enables promising DCF awards to be best positioned for Development