

Early Translational IV Concept Proposal

Presentation to ICOC

August 21, 2012

Agenda Item #3



Early Translation IV Concept Proposal In Context of CIRM Strategy



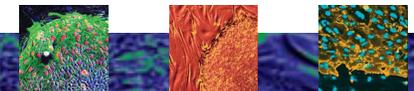
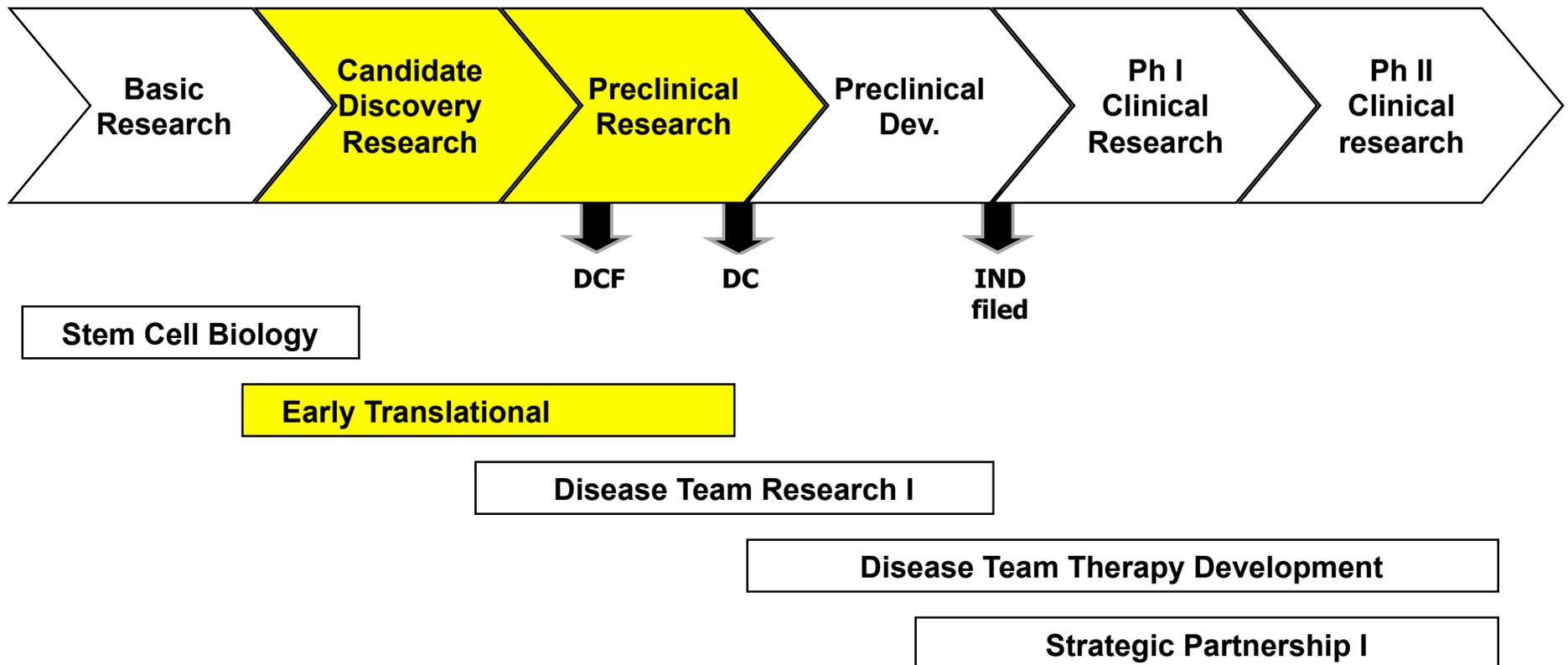
- ❑ **Scientific Strategic Objective and Key Outcome –**
Accelerate stem cell science and its applications to human diseases and injuries; achieve transformative research
- ❑ **Strategic Goals targeted by ETIV –**
 - ❑ Use RFA priorities to encourage potentially high impact, transformative research (1Yr, #3)
 - ❑ Establish proof of principle in preclinical animal models of disease or injury (5Yr, #4)
 - ❑ Creation and use of disease specific cell lines to understand pathology and discover new therapeutics (5Yr, #5)
 - ❑ Description of stem cell differentiation leading to production of critical cells (5Yr, #2)



Concept Proposal: Early Translational IV-Linking Basic Science to Preclinical Development



Goal: *To fund translation of stem cell discoveries toward IND enabling preclinical development.*



Concept Proposal: Early Translational IV - Two Types of Awards

CIRM
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

- ❑ **Development Candidate (DC) Award:** Designed to achieve a single development candidate ready for IND enabling studies
- ❑ **Development Candidate Feasibility (DCF) Award:** Designed to establish preclinical proof of concept for a potential development candidate



Concept Proposal: Responsive Projects and Priorities



Responsive Projects

- Require human stem cells
- Target endogenous human stem cells
- Propose a direct reprogramming therapeutic approach

Priorities

- Cell therapies, especially utilizing differentiated derivatives of hPSC (DC awards)
- Potentially transformative therapeutic approaches to regeneration (especially for DCF awards)



Concept Proposal: Early Translational IV – Award Eligibility



Investigator:

Principal Investigator (PI):

-Minimum 20% commitment

Co-Principal Investigator (Co-PI):

-DC awards

-Minimum 15% commitment

Institution:

-Open to non-profit and for-profit institutions

-Pre-Application process in lieu of institutional limits

Collaborative Funding Partner

-Current partner participation includes: NIH (USA), NHMRC (Australia), MOST (China), MOST (Argentina), BMBF (Germany), InSTEM (India)



Concept Proposal:

Early Translational IV – Budget and Mechanism



Development Candidate (3-year awards):

- \$3.5 MM maximum direct project costs/award
- ~10 awards

Development Candidate Feasibility (3-year awards):

- \$1.2 MM maximum direct project costs/award
- ~10 awards

Estimated total program cost up to \$70 MM

Award mechanism = grants, loan option to for-profit organizations for awards of \$3 MM or more



Concept Proposal

Early Translational IV – Time Table



Provisional Time Table

- | | |
|------------------|----------------|
| – Release of RFA | September 2012 |
| – GWG Review | June 2013 |
| – ICOC approval | Summer 2013 |



Concept Proposal: Early Translational IV – Request for Approval



- Request approval of the concept plan for the CIRM Early Translational IV Research Award with a budget of up to \$70 million

