

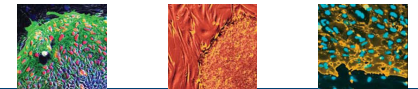
CALIFORNIA'S STEM CELL AGENCY

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Extraordinary Supplement Awards

ICOC Meeting, May 29, 2014
Agenda Item #10

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Extraordinary Supplement Awards



- In December 2012, the ICOC approved a total of \$16 MM for Extraordinary Supplements
 - \$12 MM for ‘Major’ supplements
 - \$4 MM for ‘Minor’ supplements
- Program Goal: Provide supplemental funding to existing CIRM-funded research project that would allow achievement of very significant transformational and/or translational results

Extraordinary Supplement

- **Project Eligibility and Scope**
 - Open to active* CIRM awards that
 - Are at least 1 year into project period
 - Show satisfactory progress on project
 - Scope and other requirements are those of the parent RFA
- **Award Features**
 - Funding
 - “Minor” Supplement: the lesser of 10% of the existing award or up to \$500,000
 - “Major Supplement: the lesser of 50% of the existing award or up to \$3,000,000

* Excludes the following awards: Training, Bridges, Shared Lab, Creativity and Conference Grants

Extraordinary Supplement: Major Supplement Application & Review Process



- PI submits a brief proposal that summarizes the proposed research and addresses how it meets the goal of the supplemental funding program
- Proposal assessed internally by staff for eligibility and responsiveness
- CIRM President makes decision to invite submission of a full application upon review of proposal and with staff input
- If invited, PI submits full application
- Full application is reviewed by GWG* who makes a funding recommendation
- ICOC makes funding decision

* CDAP can review for FDA mandated supplementary studies for DT or SP awards

Extraordinary Supplement: Minor Supplement Application & Review Process



- Process is identical through invitation to submit a full application
- Full application is reviewed by at least 2 members of the GWG who make a funding recommendation to the President
- The President makes funding decision with staff input
- The ICOC is notified

Extraordinary Supplement: Review Criteria

- Objective, Rationale and Responsiveness
 - Outcomes potentially transformational
 - Not achievable with existing funding
- Feasibility and Design
 - Existing Project Progress
 - Preliminary Data
 - Research Plan
 - Budget
- Team, Assets, Collaborations, Resources and Environment

ES1-01249 – Minor Supplement Notification

- Request for minor supplement funding to TR1-01249 to conduct studies to assess process scalability of therapeutic candidate as well as preclinical studies in target indication of osteonecrosis
- Requested funding: \$499,868
- Reviewed by 3 members of the GWG all of whom strongly recommended funding
- President approved

ES1-05501 – Major Supplement

- Request for major supplement funding to TR3-05501 to evaluate human skeletal muscle stem cells in an additional indication (stress urinary incontinence) that could offer a potentially faster path for clinical development and be potentially transformative
- Requested funding: \$900,424
- Reviewed by GWG (major supplement)
 - Average score = 73 → Tier 2

ES1-05501 – Major Supplement

- Staff recommendation: Fund
 - leverages team expertise and know-how to address preclinical proof of concept for a second disease target where the number of engrafted cells required for disease modification is low
 - Complementary approach for portfolio: skeletal muscle stem cells vs smooth muscle stem cells.