CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

The state stem cell agency

Early Translational Awards III Awards

RFA 11-02
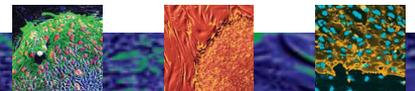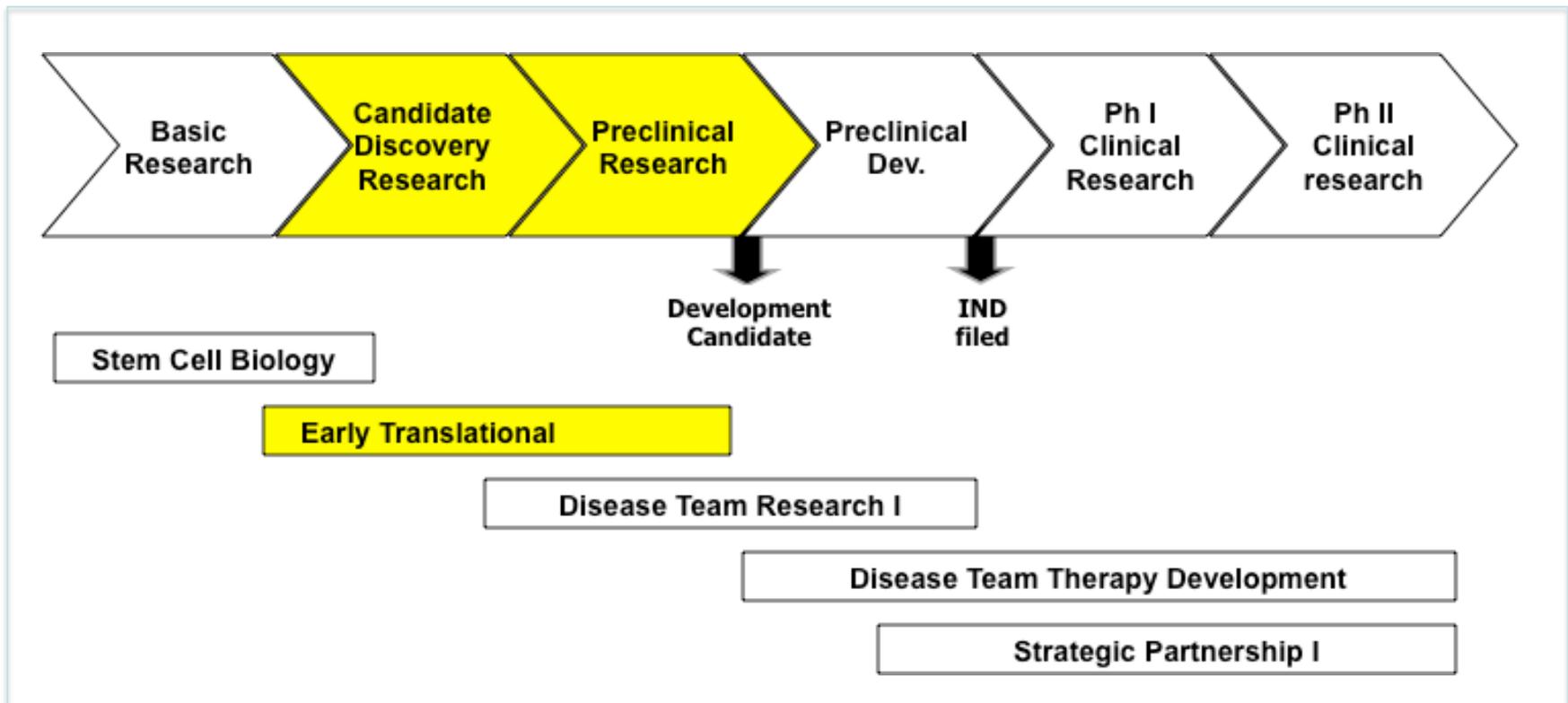# ICOC Meeting May 24 2012
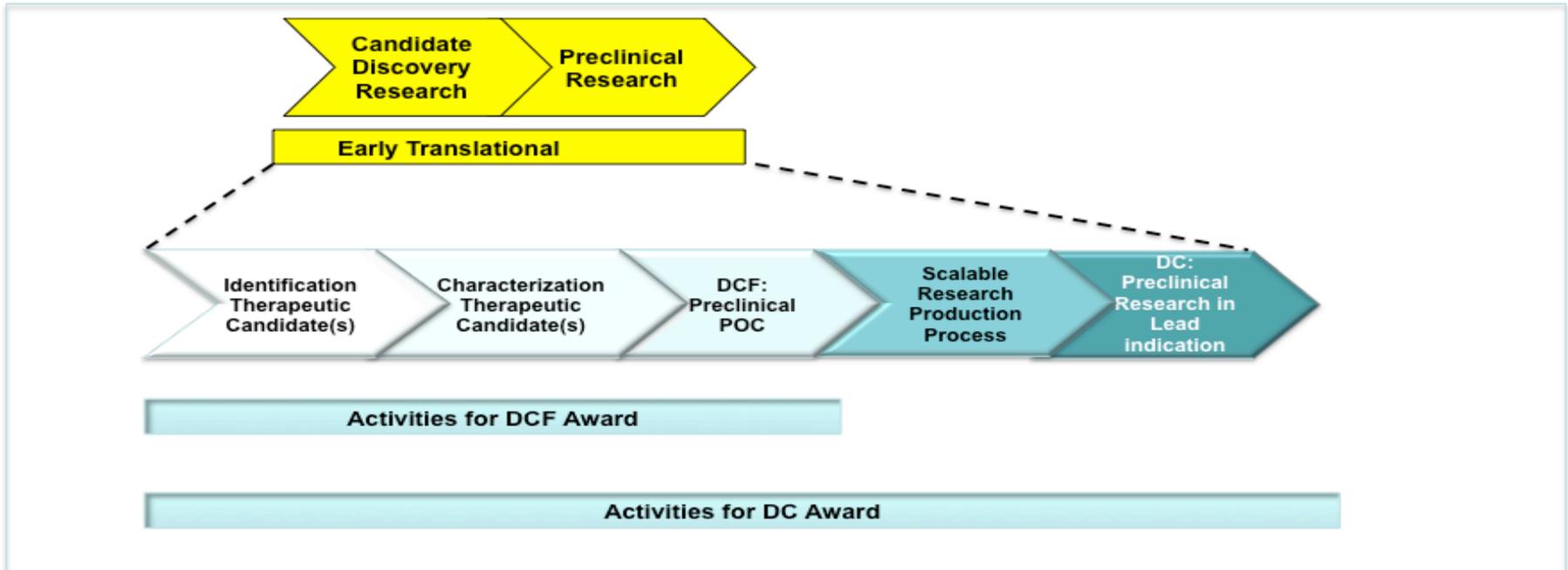# Agenda Item #7

Early Translational III
Linking Basic Science to Preclinical Development

Goal: *To advance translational stem cell science toward IND enabling preclinical development.*

Early Translational III - Two Types of Awards

- ❏ **Development Candidate (DC) Award:** To advance therapeutic candidates up to the IND enabling stage

- ❏ **Development Candidate Feasibility (DCF) Award:** To advance therapeutic candidates through initial proof of concept

Early Translational III – Award Priorities

❑ Programs using cell therapy:

✓ Especially cells that are differentiated derivatives of pluripotent stem cells (PSC)

❑ Programs using derivatives of pluripotent stem cells to identify therapeutic candidates

If represented in CIRM's translational portfolio novel approaches required

Early Translational III – Scope

In-Scope

❑ Identification of therapeutic Candidates (i.e. stem cells, mAb, Small molecule)

❑ Characterization of therapeutic Candidates in-vitro and/or in-vivo

❑ Research production scale

❑ Disease modifying activity

❑ Disease in a dish

Out of Scope

❑ Basic Research

❑ Drug Target Discovery

❑ GMP Production

❑ IND Enabling preclinical studies

  ✓ GLP Toxicology

  ✓ GLP Tumorigenicity studies

❑ Clinical studies

Early Translational III: Review Criteria

1. Objective and Milestones
2. Rationale and Significance
3. Research Project Feasibility and Design
4. Qualification of PI, CO PI, Partner PI and Research Team
5. Collaborations, assets, Resources and Environment
6. Responsiveness to the RFA

Early Translational III: Review Criteria

1. Objective and Milestones:

❑**Objective**: Achieving DC or DCF

❑**TPP**: Scientifically and clinically reasonable

❑ **Milestones:** quantifiable, address key requirements to achieve either DCF or DC

Early Translational III: Review Criteria

2. Rationale and Significance

❑**Scientific rationale**: Logical and compelling for the target for intervention

❑**Significance**: Address unmet medical need and could lead to a significant improved therapy for the proposed disease&injury

Early Translational III: Review Criteria

3. Research Project Feasibility and Design:

❑ **Preliminary Results**: Compelling and supportive of the proposed research

❑ **Research Plan**: Focused, well designed to achieve milestones and objective within three years

Early Translational III: Review Criteria

4. Qualifications of PI, (Co-PI, Partner PI) and Research Team:

❑**Training and Experience:** The PIs and Co-PI have experience in the relevant translational science.

❑**Track Record**: The PIs and Co-PI have a successful record in the proposed translational project

❑**Appropriate Team:** Appropriate multidisciplinary research team

❑**Team Communication**: Reasonable coordination and communication plan

❑**Commitment and appropriate budget**

Early Translational III: Review Criteria

5. Collaborations, Assets, Resources and Environment:

❑ **Collaborations:** are critical and integral part of the proposed project

❑ **Relevant Assets**: IP, MTAs and reagent material are available for the project

❑ **Resources and Environment:** Availability of appropriate facility and equipment

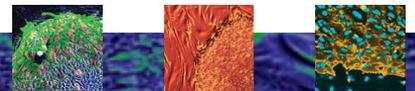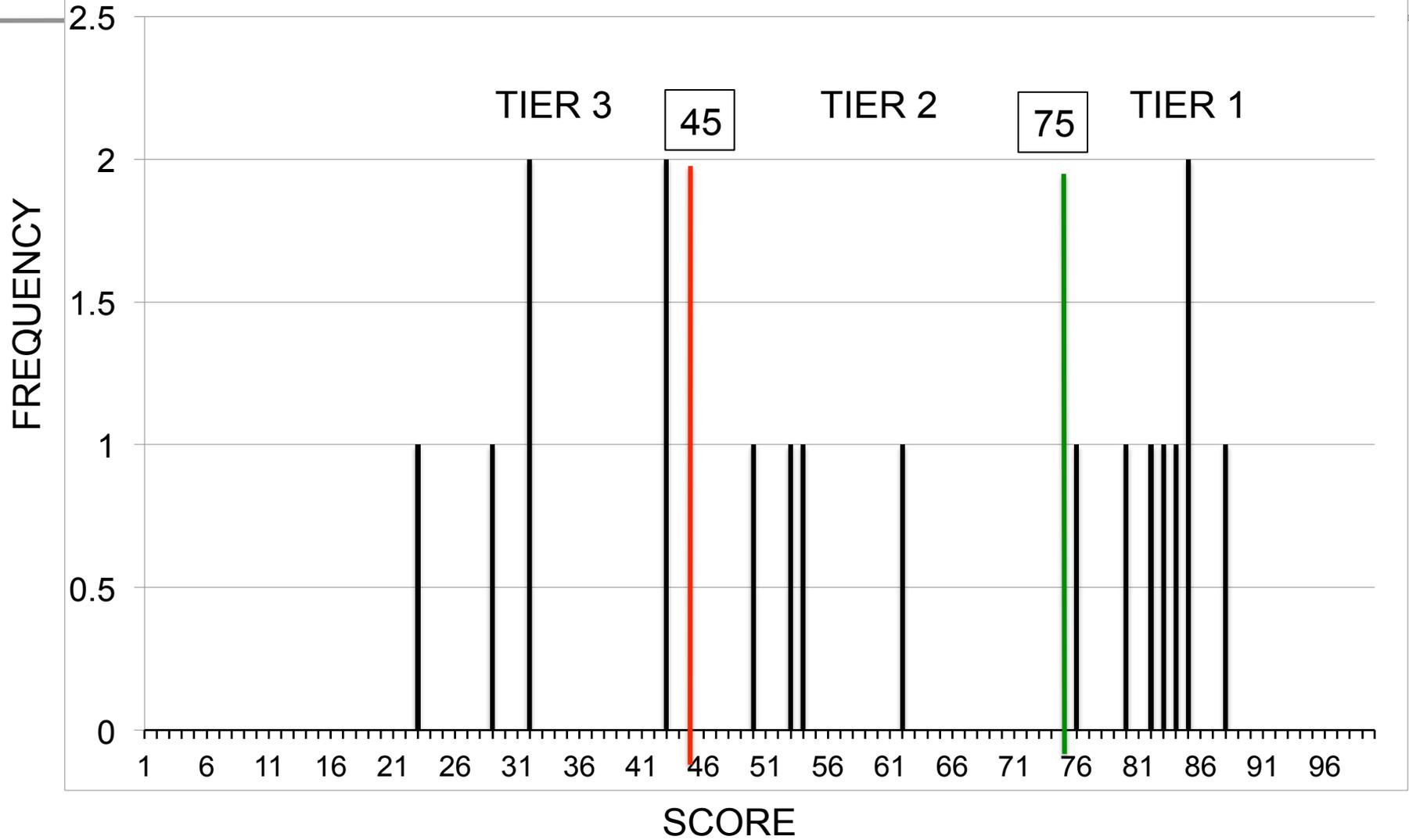❑ **Institutional Support**: Institution is committed to support the project

Early Translational III: Review Criteria

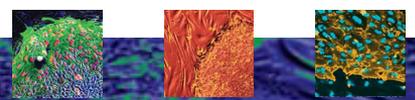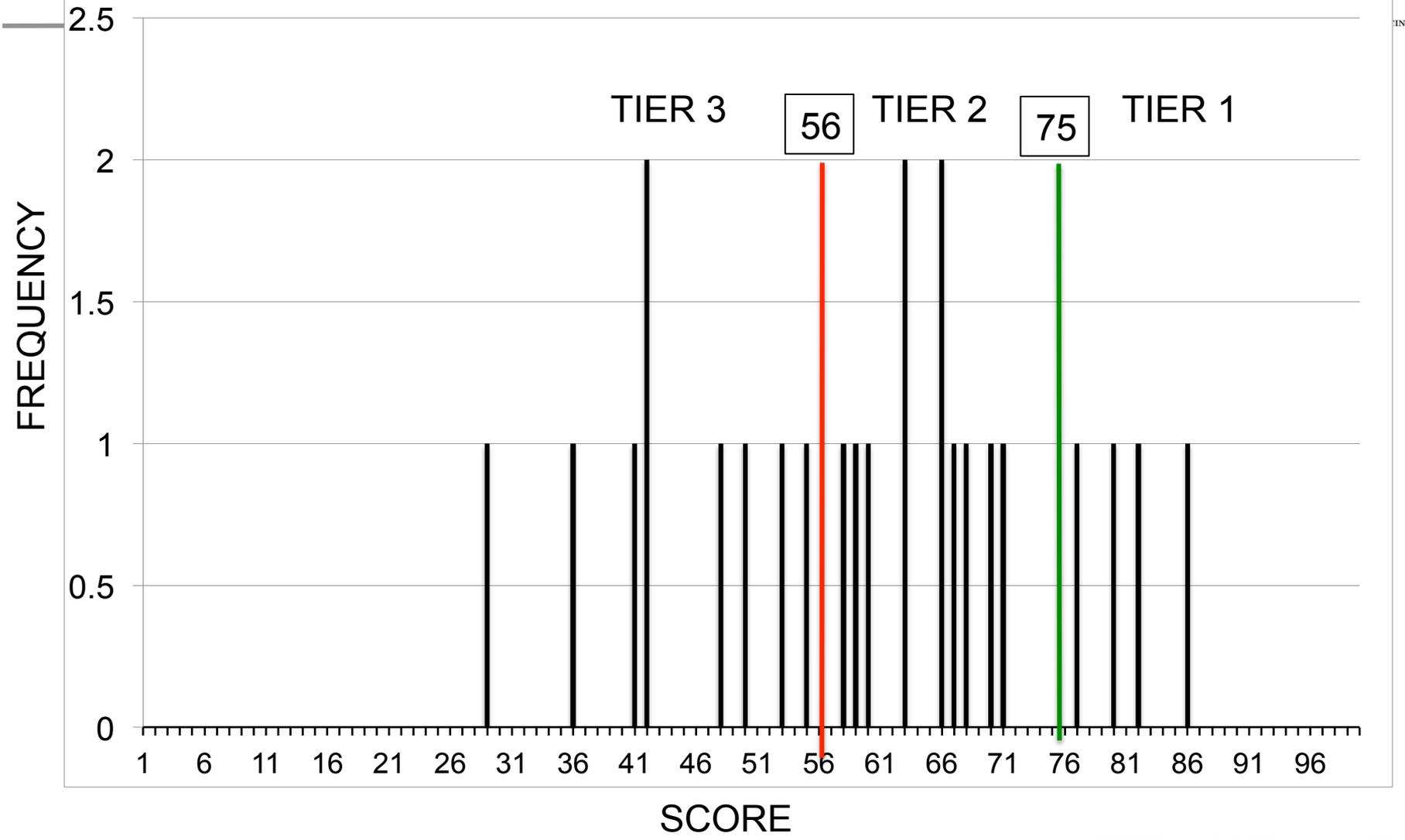CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

6. Responsiveness to the RFA:

❑Addresses the RFA objectives

❑Use of human stem cells for POC and Development Candidate

❑Identified a target for intervention (DC award only)

❑Portfolio consideration

DCF Score Distribution

TIER 3   45   TIER 2   75   TIER 1

DC Score Distribution

TIER 3 | 56 | TIER 2 | 75 | TIER 1

FREQUENCY

SCORE

GWG Recommendations

	DC	DCF	Total
Target Number of Applications	15	10	25 ($95M)
Recommended for Funding	9*	10	19 ($62.8M)

*One of the DC application was recommended for funding as a DCF