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# M E M O R A N D U M

**March 3, 2014**

**From:** Lisa Kadyk, Ph.D., Science officer
Patricia Olson, Ph.D., Executive Director of Scientific Activities

**To:** Independent Citizens Oversight Committee (ICOC)

**Subject:** Concept proposal for RFA 14-02: Preclinical Development Awards

This concept proposal addresses a new initiative that is proposed to facilitate the advancement of promising therapeutic candidates towards the clinic. Applicants will have completed all research activities necessary to identify a single therapeutic candidate to move into development (a Development Candidate). This award will fund key early development activities necessary for a productive preIND meeting with the Food and Drug Administration (FDA). Such activities include development of a good manufacturing process (GMP), assay development and qualification, optimization of dosing and route of administration, mechanism-of-action studies, development of a clinical plan, and holding a well-prepared preIND meeting with the FDA to discuss the IND-enabling development plan. Completion of the activities in this award should position a project on the development pathway to be successful in IND filing and moving into Phase 1 clinical trials for patients, to attract industry partners and to be competitive for future funding.

## Concept Proposal for RFA 14-02: Preclinical Development Awards

*Program Goal:* To support early preclinical development of promising Development Candidates, positioning them for successful IND filing and Phase 1 clinical trials, adding value to them for partnering or for accessing other funding.

*Award Amounts*:

- $40M total allocation for 4-5 awards
- Up to $8M/award ($10M in extraordinary circumstances)

*Award mechanism:*

- Grant, for a not-for-profit organization, *or*
- Choice of Grant or Loan, for a for-profit applicant organization.

*Objective*: Completion of a successful preIND meeting with the FDA within 30 months.

*Eligibility*:

- Projects for which CIRM has funded prior early translational work on the intended therapeutic candidate, *or*
- New projects that have industry partnerships.

*Readiness*: Single therapeutic development candidate selected, with strong reproducible evidence for disease-modifying activity by the proposed candidate in the target (or related) disease/injury.

**Provisional Time Table:**

| | | |
|---|---|---|
| Posting of RFA 14-02 | May | 2014 |
| LOI applications due | June | 2014 |
| Full Applications due | August | 2014 |
| Review of Applications by Grants Working Group (GWG) | Q4 | 2014 |
| Review and Approval by ICOC | Q1 | 2015 |
| Earliest Funding of Awards | Q2 | 2015 |

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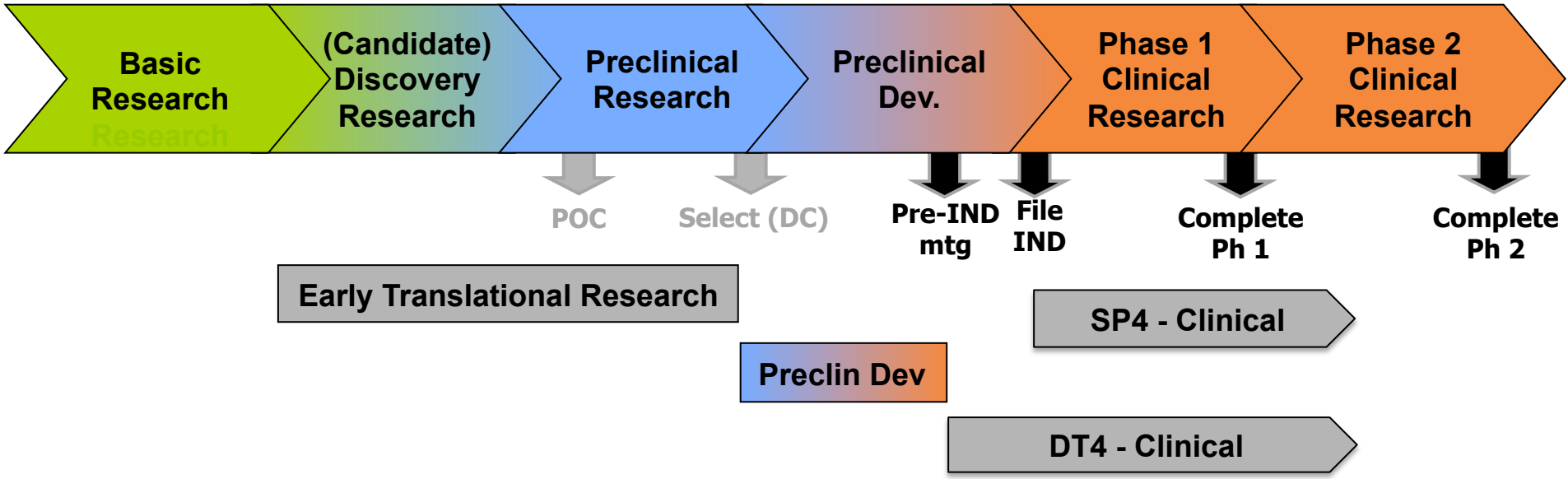
*The state stem cell agency*

# Preclinical Development Awards Concept Plan PreRead

ICOC Meeting March 11, 2014
Agenda Item # 7

# Purpose and Scope:
## Preclinical Development Awards




- New award would fund early development activities needed to transition between identification of a Development Candidate and conduct of a pre-IND meeting.

- Goal is to position promising projects to have successful IND filings, attract partners and/or be competitive for future funding.

# Goal & Objective:
## Preclinical Development Awards

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- Program Goal: Initiate development with promising existing or new (if partnered with industry) therapeutic candidates
- Project Objective: Completion of a well-prepared **pre-IND meeting** with the FDA within **30 months**
  - Rationale:
    - Requires critical FDA input on project which helps to define plans and readiness for future development.
    - Limits investment for early development work. Pivotal IND-enabling activities (GLP safety studies, GMP manufacture of clinical therapeutic candidate, IND filing and Phase 1 clinical studies) would require new funding.
    - Facilitates external interest by potential partners.

# **Strategic Alignment:** Preclinical Development Awards

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- Aligned with focus of CIRM's 2012 strategic plan:
  - To advance stem cell science toward clinical trials that have the potential to generate evidence of therapeutic benefit to patients.
  - To leverage CIRM's investment through partnership with industry.

# **Eligibility:** Preclinical Development Awards

- **CIRM funded** earlier translational work on the intended therapeutic candidate

*OR*

- **New project** if have a research/development agreement with a large biotech/pharma partner or can show strong evidence of partner interest at time of application (e.g research collaboration term sheet).
  - Partner may provide co-funding or in-kind support
  - Signed agreement required before approval by ICOC

- Applicant may be for-profit or not-for-profit

# **Readiness:**
# Preclinical Development Awards

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- **Single** therapeutic development candidate selected
- Strong, reproducible evidence for preclinical **disease-modifying activity** for the proposed candidate in target (or related) disease/injury
- Preliminary assessments of dose/safety
- Research scale production/assays in place

# Activities:
## Preclinical Development Awards

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### *In-Scope*

- ❑ Development of a stage-appropriate GMP manufacturing process and assay development/qualification
- ❑ Optimization of dose, route of administration in models
- ❑ Mechanism of Action studies
- ❑ Development of a clinical plan
- ❑ Conducting pre-preIND and preIND meetings

### *Out of Scope*

- ❑ Research to identify a therapeutic candidate
- ❑ Studies to explore other non-related indications
- ❑ Pivotal IND-enabling safety studies
- ❑ Manufacture of clinical supplies of therapeutic candidate

# **Therapeutic Candidates:** Preclinical Development Awards

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## *In-Scope*

- ❑ Pluripotent-cell derived
- ❑ Allogeneic or autologous adult stem or progenitor cells (with exceptions)
- ❑ Genetically- or pharmacologically modified HSC or MSC
- ❑ Tissue-engineered tissues
- ❑ Small molecule or biologic that targets normal endogenous stem cells

## *Out of Scope*

- ❑ Unmodified HSC or MSC
- ❑ Minimally manipulated bone marrow or cord blood cells
- ❑ Small molecules or biologics not targeting endogenous stem cells

# **Award Information:**
# Preclinical Development Awards

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- Total Program Costs: up to $40 MM
  - Estimate 4-5 awards
- Award Amount
  - $8 MM per project
  - Under exceptional circumstances up to $10 MM
- Award Term
  - 2.5 years (30 months)
- Award mechanism
  - Grant (non-profit applicant organization)
  - Grant or Loan (for-profit applicant organization)

# Provisional Time Table:
## Preclinical Development Awards

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| ET Preclinical Development Provisional Time Table | | |
|---|---|---|
| Post RFA 14-02 | May | 2014 |
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# **Request for Approval:** Preclinical Development Awards

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- Request approval of the concept plan for the CIRM Preclinical Development Awards RFA 14-02 with a proposed budget of $40 MM.

*Questions?*