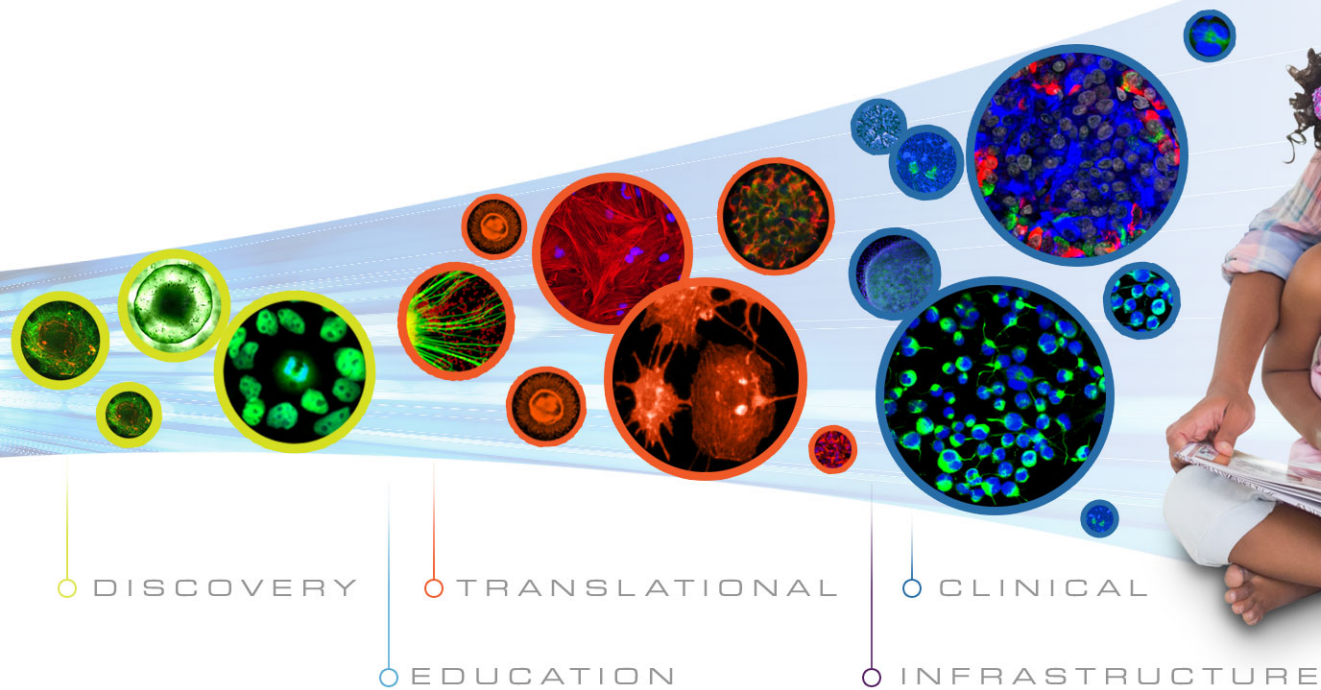


CIRM

CALIFORNIA'S STEM CELL AGENCY



CLIN, TRAN and DISC Concept Plan Amendments

Gil Sambrano

Vice President, Portfolio Development and Review

Good Standing Requirement (All concepts)

Require applicants to verify:

- (1) systems in place to track CIRM funds (*limited to for-profits and non-profits in existence for < 5 years*)
- (2) CEO, CFO, or PI has not been convicted of, and is not under investigation for, crimes involving fraud or misappropriation (*limited to for-profits and non-profits in existence for < 5 years*)
- (3) PI is not under investigation for research misconduct and is not barred from receiving research funds by DHHS Office of Research Integrity

Personnel Eligibility

- **Project Manager:** Allow applicants to satisfy requirement by entering into a contract with CIRM's Stem Cell Center (CLIN and TRAN 1-3)
- **PI Percent Effort:** Allow PI to propose/justify percent effort consistent with achieving the project's aims rather than requiring 30% (CLIN)

Project Eligibility

- **Readiness:** Reduce time to IND filing eligibility requirement for CLIN 1 applicants from 24 months to 18 months
- **Small Molecule/Biologic:** Clarify eligibility of research involving small molecules or biologics for which a stem cell is necessary to manufacture the therapy (TRAN 1, CLIN 1 and CLIN 2-Phase 1 trials)

Project Eligibility

- **Phase 2 Trials:** Restrict to cellular therapies where stem/progenitor cells either compose the therapy or are used to manufacture the cell therapy
- **Phase 3 Trials:** Restrict to cellular therapies where stem/ progenitor cells either compose the therapy or are used to manufacture the cell therapy AND where the therapy is for rare indications
- **CLIN 3:** Limit to awardees that are able to convert an existing trial to a registration trial

Eligibility for Devices

- **TRAN 3:** Include studies on a device where the therapeutic mechanism of action requires the recruitment or incorporation of an endogenous human stem or progenitor cell
- **CLIN 2:** Limit device trials to feasibility studies

Funding Caps (CLIN)

- CLIN 1: \$6M (non-profits) & \$4M (for-profits)
- CLIN 2: (1) Phase 1 trial: \$5M (for-profits) and \$9M (non-profits)
(2) Phase 2 trial: \$12M
(3) Phase 3 trial: \$15M
- CLIN 3: \$15M

Fundable Activities

- CLIN 1 and 2: Permit funding for manufacturing activities for a follow-on clinical trial
- CLIN 2: Permit funding for comparability studies and commercial development activities

REQUESTED ACTION

Recommend Board approval of proposed amendments to CLIN, TRAN and DISC concept plans