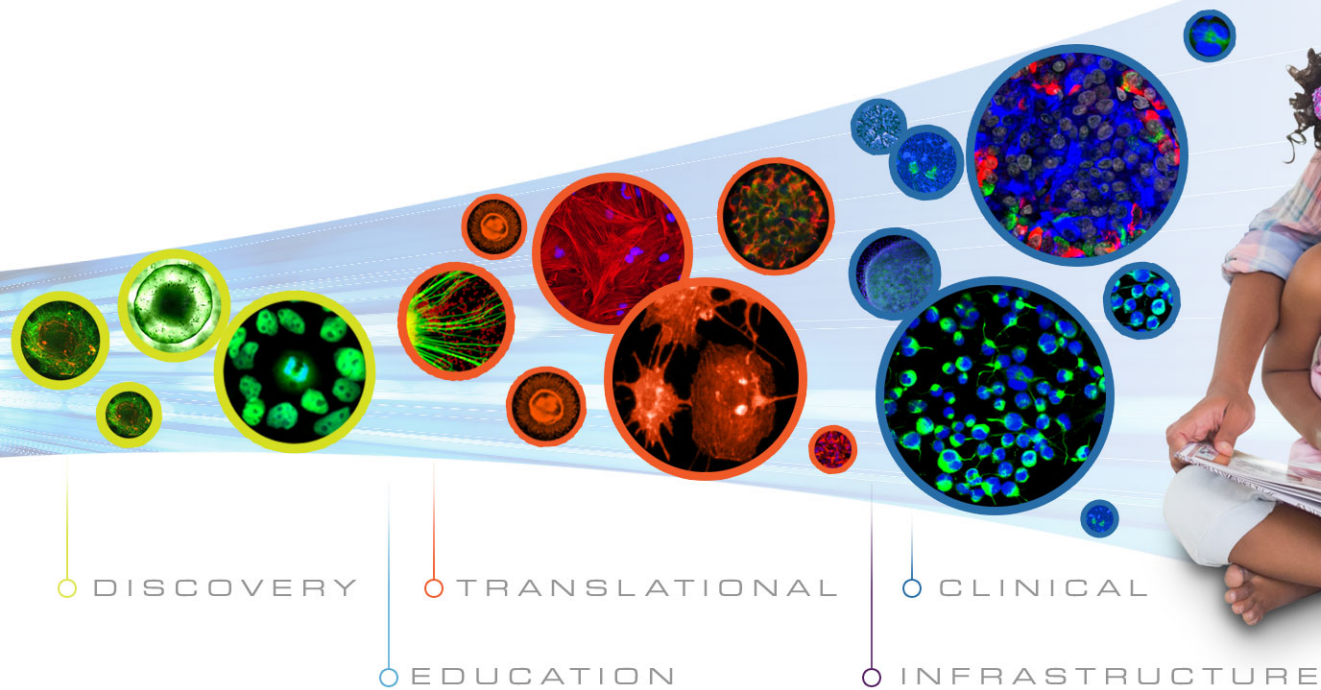


CIRM

CALIFORNIA'S STEM CELL AGENCY



CIRM 2.0 for Discovery & Translation Proposed Concept Plans

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Topics



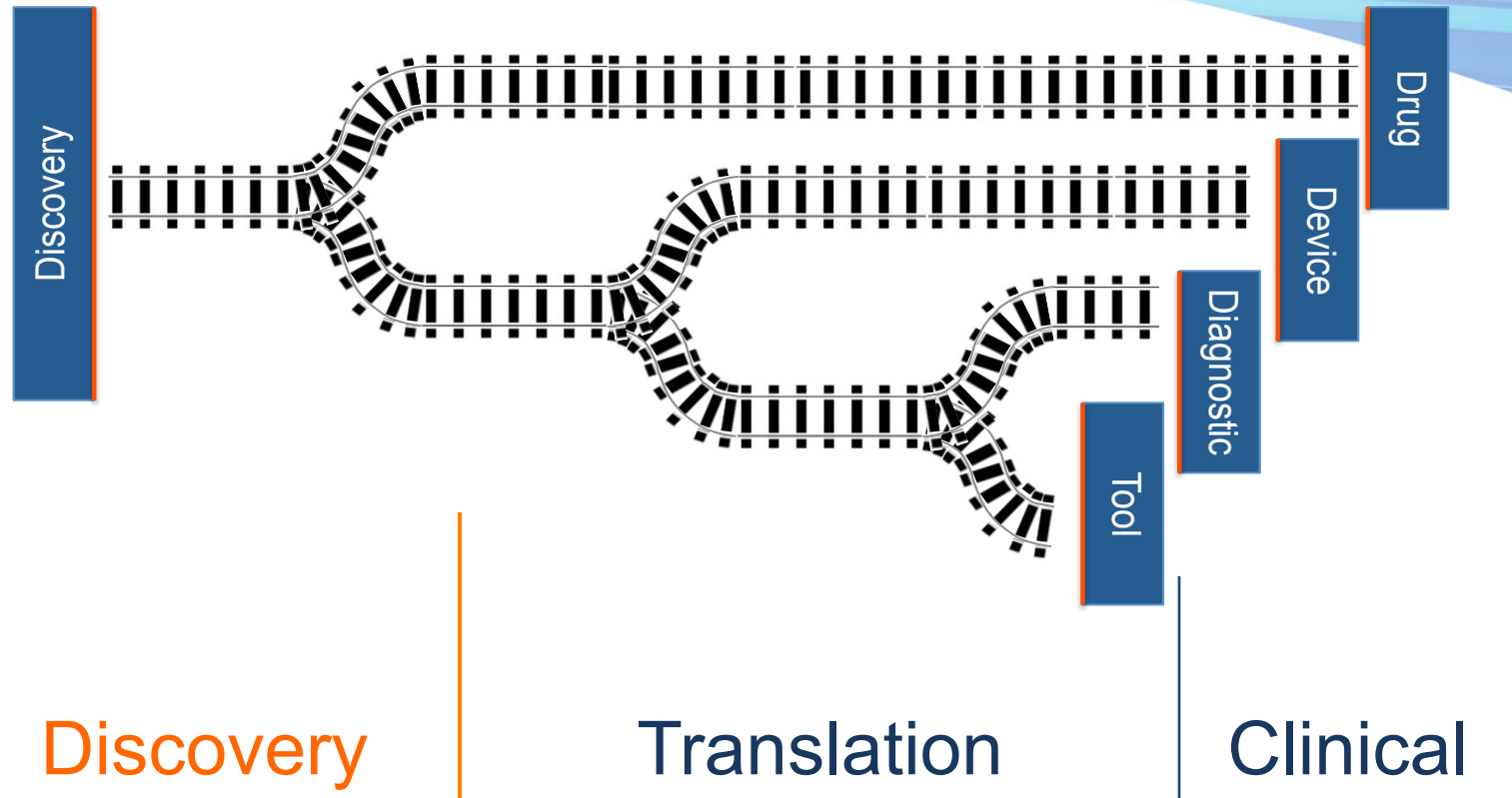
DISCOVERY
TRANSLATIONAL
CLINICAL
EDUCATION
INFRASTRUCTURE

- CIRM 2.0 **Discovery** Stage Concept Plan
 - Optimized Review Process for High Volume Applications

- CIRM 2.0 **Translation** Stage Concept Plan

CIRM 2.0 Discovery Stage

Concept Plan



CIRM 2.0: Discovery Stage Concept

Three New Programs

Inception Awards



- Smaller seed funding
- Exploration of transformational ideas
- Generate early data for larger, follow-on awards
- Links to Quest Awards

Quest Awards



- Early research on new stem cell ideas or concepts
- “Workhorse” of Discovery
- Incentives for progression of successful programs
- Links to Translation Awards

Challenge Awards



- Timely calls to address highly specific topics
- Address emerging concepts, bottlenecks or downstream needs

Inception Awards

CIRM 2.0: Discovery Stage Programs

- **Purpose:** To provide seed funding for potentially transformational but very early stage concepts involving stem cells
- **Investment:** \$150,000 direct costs per project (est. \$250,000 fully burdened)
- **Duration:** Not pre-specified, proposed and justified by applicant
- **Progression:** Applicant must provide a vision of a successful outcome and subsequent progression steps
- **Volume:** High application volume expected

Quest Awards

CIRM 2.0: Discovery Stage Programs

- **Purpose:** To discover a novel candidate therapeutic, device, diagnostic or tool and prepare it for translational research
- **Investment:** \$1.4M in direct costs per project if a therapeutic (est. \$2.25M fully burdened); \$700,000 if a diagnostic, device or tool (\$1.1M fully burdened)
- **Duration:** Up to 2 years
- **Progression:** Applicant must provide a vision of a successful outcome and subsequent progression steps; incentive award offered for successful progression
- **Volume:** High application volume expected

Quest-Progression Awards

CIRM 2.0: Discovery Stage Programs

- **Purpose:** To increase the efficiency at which promising Discovery research progresses into the translational stage and to reinvest in Discovery stage researchers that successfully advance research
- **Investment:** \$150,000 direct costs per project (est. \$250,000 fully burdened)
- **Duration:** Not pre-specified, proposed and justified by applicant
- **Progression:** Applicant must provide a vision of a successful outcome and subsequent progression steps
- **Volume:** Only open to Quest Awardees submitting documentation that successful funding for translational stage research has been achieved. CIRM will verify documentation and proposal.

Challenge Awards

CIRM 2.0: Discovery Stage Programs

- **Purpose:** To address highly specific questions on emerging concepts, bottlenecks or downstream needs as requested by CIRM or the ICOC
- **Investment:** As appropriate, up to \$2 million
- **Duration:** As appropriate for the topic
- **Progression:** Applicant deliverable must be responsive to the question.
- **Volume:** Low application volume expected



OPTIMIZED REVIEW PROCESS FOR HIGH VOLUME PROGRAMS

Optimized Review Process

CIRM 2.0: Discovery Stage Programs

For rounds with high application volume, a two stage review will be implemented:

- **Stage 1:** A pre-review will be conducted by the 22 members of the GWG (15 Scientific, 7 Patient Advocate) to select a subset of applications to advance to Stage 2 of review. CIRM will also have an opportunity to select applications for review.
- **Stage 2:** Standard, in-person GWG review meeting resulting in funding recommendations to the ICOC.

This process improvement offers:

- Single step application process (will eliminate the “Pre-App” process)
- Is scalable in response to application volume
- Can facilitate selection of proposals with high potential for impact from a broad applicant pool



CIRM 2.0 DISCOVERY CONCEPT PLAN PROPOSED ACTION

Proposed Action Items

CIRM 2.0: Discovery Stage Programs

Delegation of Board authority to President or designee to accomplish the following:

- To examine and select other applications that merit second stage review beyond those selected by the GWG (if any) and to determine not to fund the remaining applications
- To determine if a Progression Event has been achieved under a Quest Award, evaluate Quest-Progression proposals, and make conforming awards
- To issue timely Challenge Question Announcements that define a specific question and corresponding award terms

AND

Proposed Action Items

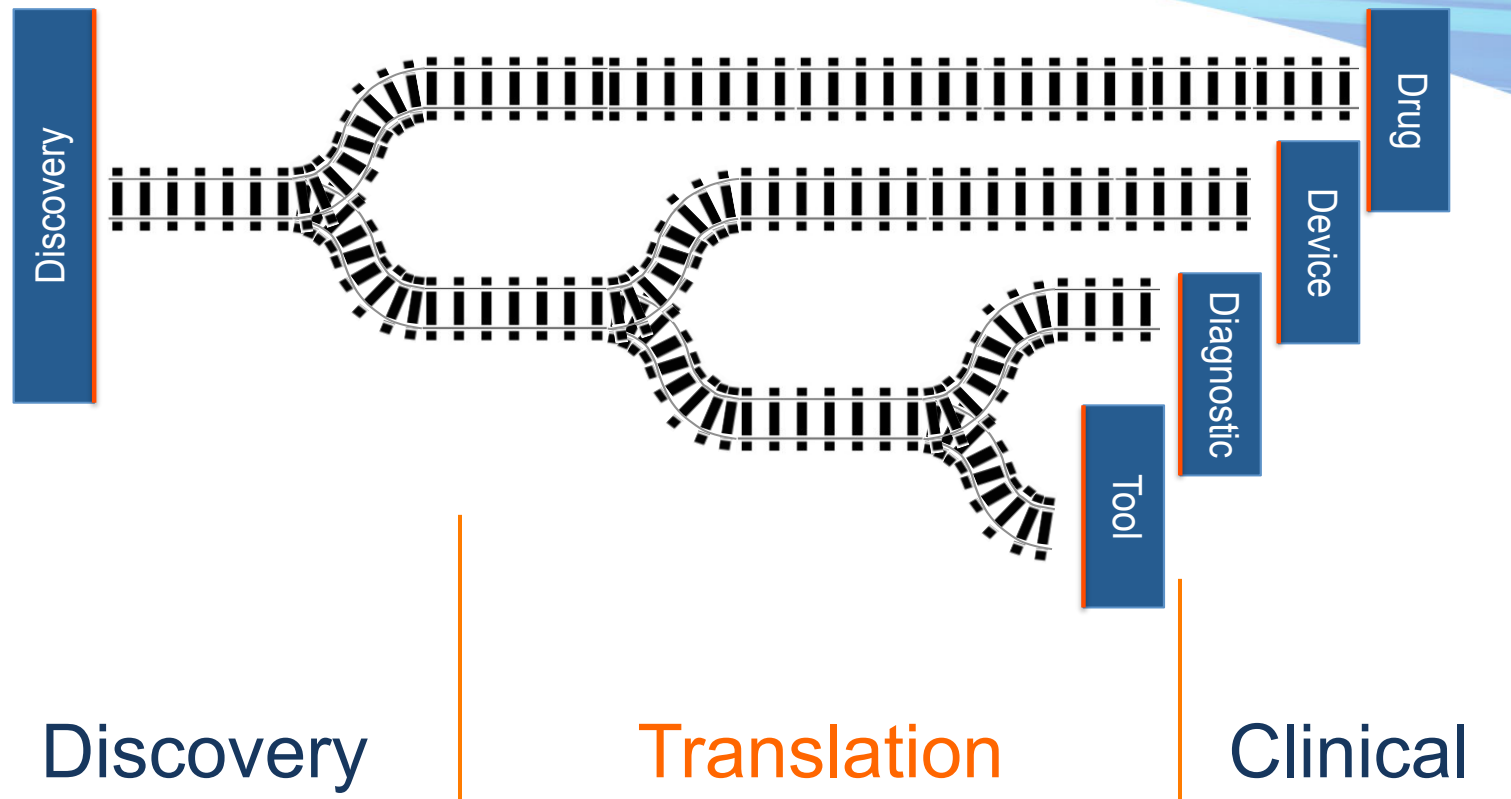
CIRM 2.0: Discovery Stage Programs

\$53M funding authorization for the Discovery Stage Concept Plan with the following allocation estimates:

Programs	Number of Awards	Investment
Inception	26	\$6.5 M
Quest / Progression	20	\$42.5M
Challenge	4	\$4 M

CIRM 2.0 Translation Stage

Concept Plan



CIRM 2.0: Translation Stage Concept

Four Specific Programs

Translation
(All “Link From” Quest Awards)

Therapeutic

Outcome links to
CIRM Clinical
Program

Device

Outcome links to
CIRM Clinical
Program or is
readiness for final
studies for regulatory
filing

Diagnostic

Outcome is readiness
for clinical or other
studies for regulatory
filing or for reference
lab commercial use

Tool

Outcome is readiness
for final studies for
manufacturing &
commercialization or
broad use

CIRM 2.0: Translation Stage Concept

Four Specific Programs

Therapeutics

- Direct Project Costs up to:
 - \$5M for cell therapy candidate
 - \$2.5M for small molecule candidate
- Project term up to 30 months

Devices

- Direct Project Costs up to \$2M
- Project term up to 24 months

Diagnostic Tests

- Direct Project Costs up to \$1.2M
- Project term up to 24 months

Tools

- Direct Project Costs up to \$1M
- Project term up to 24 months



CIRM 2.0 TRANSLATION CONCEPT PLAN PROPOSED ACTION

Proposed Action Items

CIRM 2.0: Translation Stage Programs

Delegation of Board authority to President or designee to accomplish the following:

- To examine and select other applications that merit second stage review beyond those selected by the GWG (if any) and to determine not to fund the remaining applications

AND

\$40M funding authorization for the Translation Stage Concept Plan.