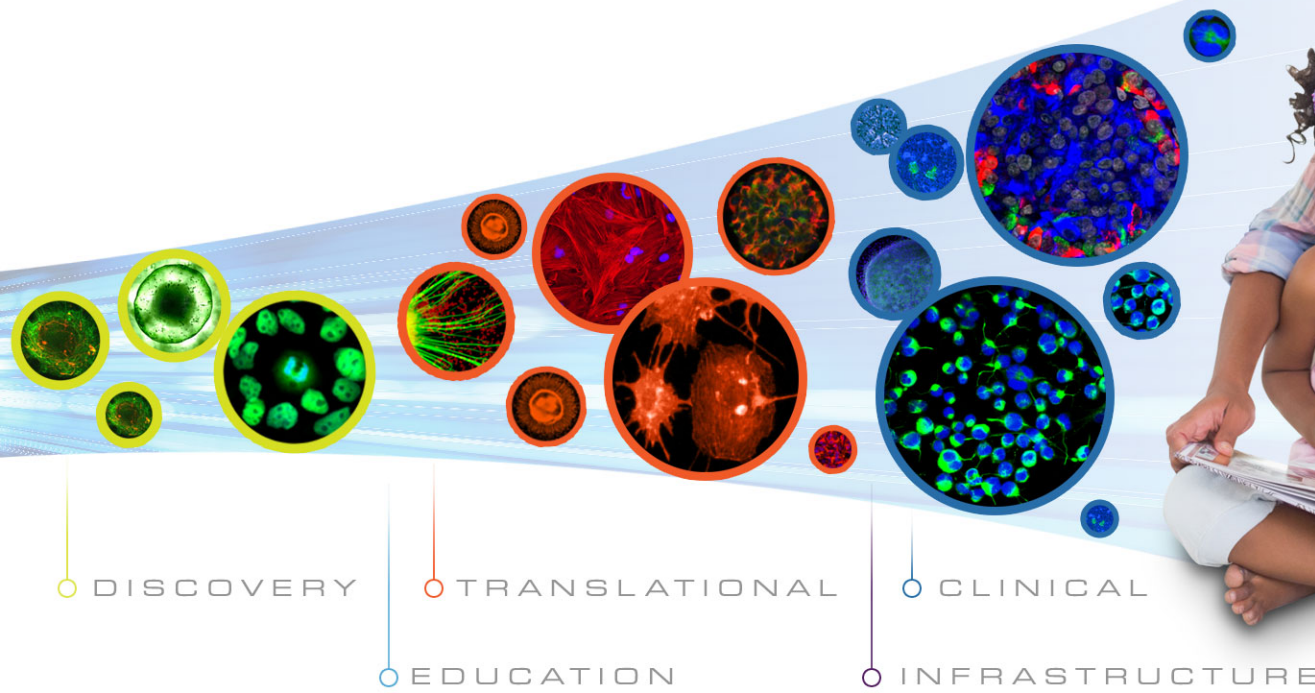


July 15, 2015

CIRM

CALIFORNIA'S STEM CELL AGENCY



CIRM 2.0

Discovery, Translation & Education Concepts

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California Institute for Regenerative Medicine

Topics



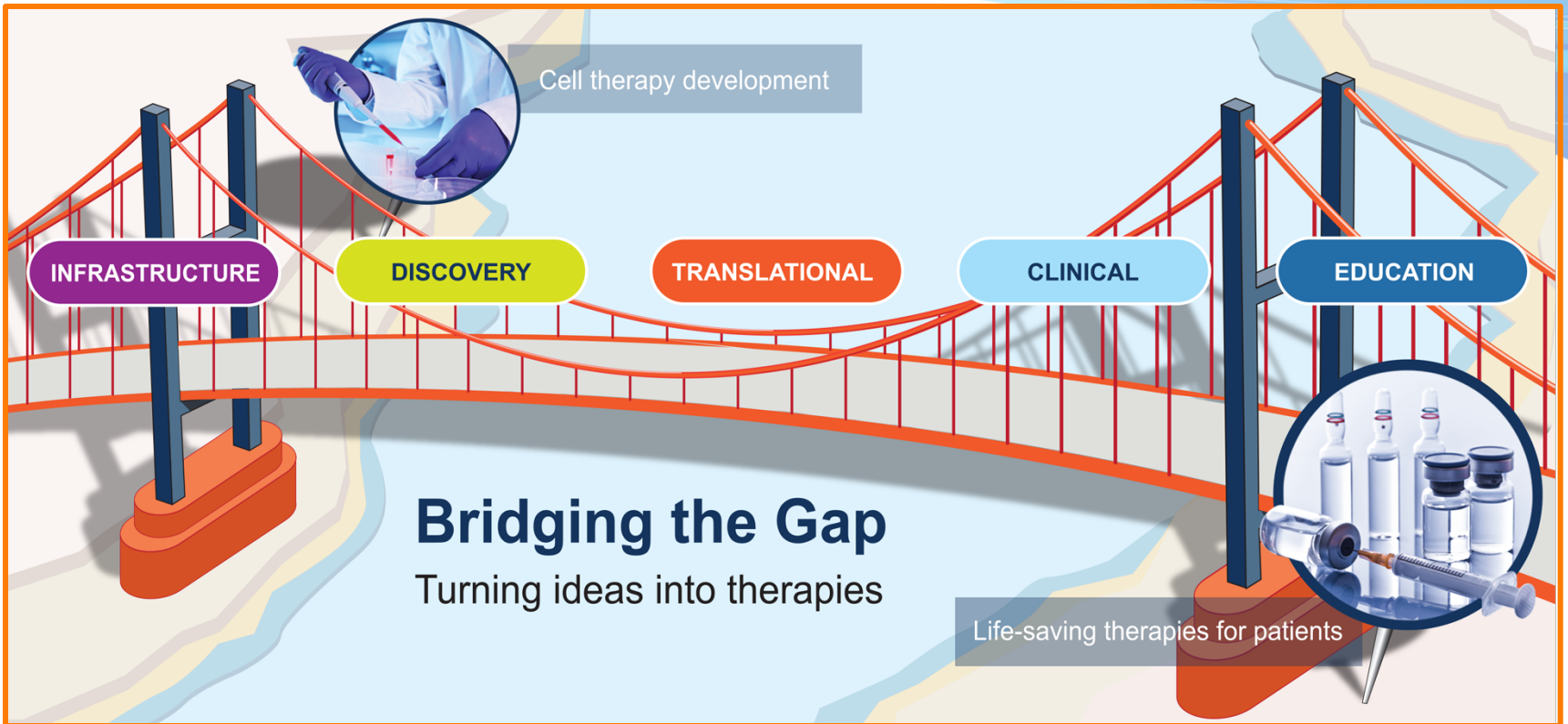
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Our Mission

Accelerating stem cell treatments to patients with unmet medical needs.

The CIRM Spectrum



CIRM 2.0 Discovery and Translation

Objective: To promote the discovery of promising new stem cell technologies and drive their rapid translation towards improving patient care

CIRM 2.0 will Improve Discovery & Translation in Four Key Areas



Continuous, Predictable
Pathway



Prescribed Progression



Responsive

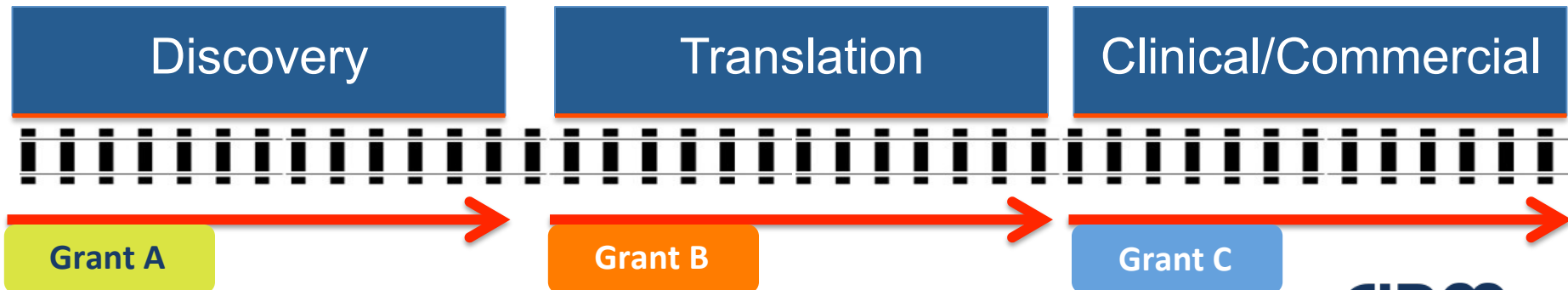


Multiple Development
Pathways

CIRM 2.0 Discovery & Translation: Continuous, Predictable Pathway

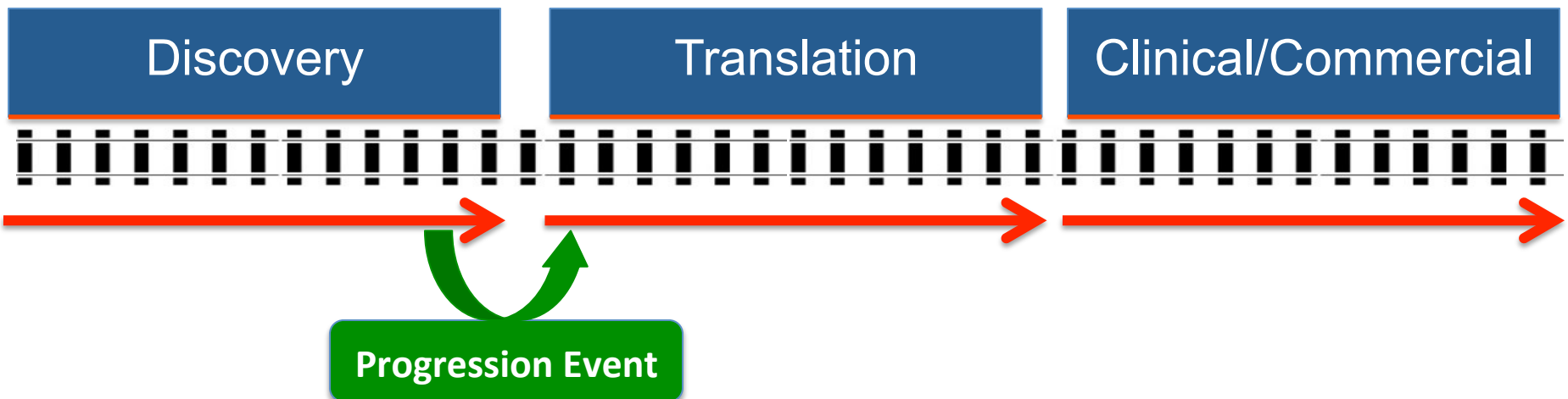


- Lay a continuous track of funding opportunities from Discovery through Clinical
- The Outcome of Grant A is the entry point for Grant B
- Regular, Recurring Calls for Applications

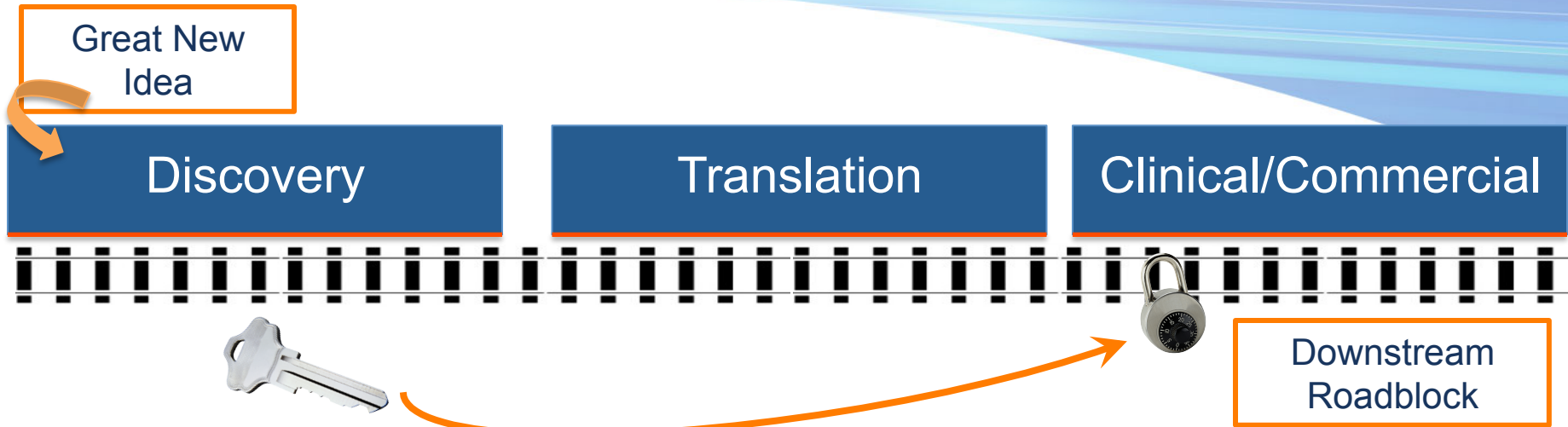


CIRM 2.0 Discovery & Translation: Prescribed Progress

- Expect and incent progression of discovery outcomes to next stage of development
- Incent progression by rewarding teams who do so, or who “pass the baton” to another team who will translate

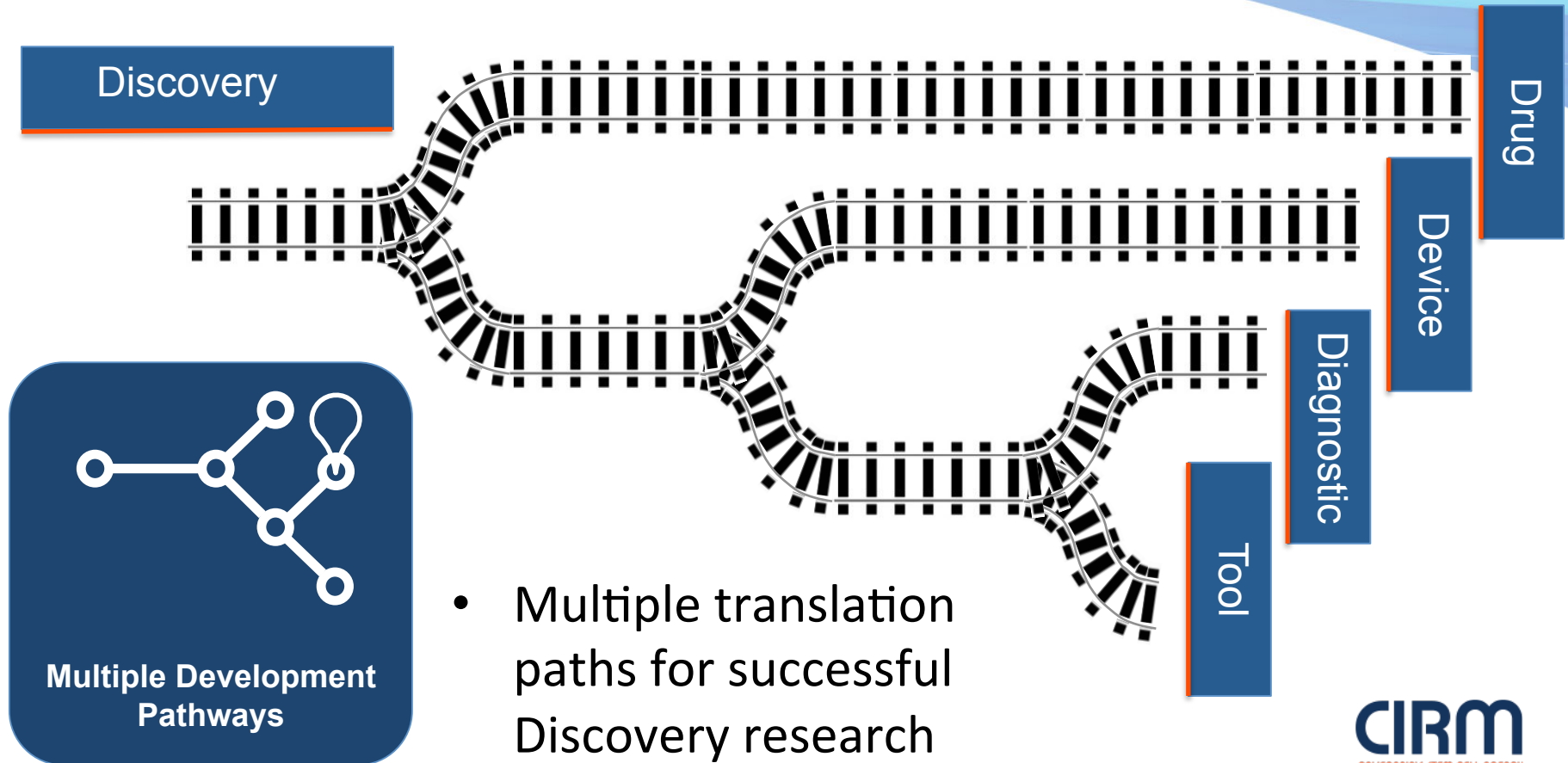


CIRM 2.0 Discovery & Translation: Timely, Responsive



- Timely and responsive to downstream needs, emerging concepts and great new ideas

CIRM 2.0 Discovery & Translation: Multiple Development Pathways



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CIRM 2.0: Discovery Stage Programs

Three New Programs

PA 15-07: Inception Awards

- Exploration of great new ideas
- Generate rationale for new discovery

PA 15-08: Quest Awards

- Discovery research on new stem cell technologies to ready for translation
- Incent progression to translation
- Enrichment activities for students and fellows

PA 15-09: Challenge Awards

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

CIRM 2.0: Discovery Stage Programs: PA 15-07: **Inception Awards**

- Goal is to test a novel idea that could lead to discovery of promising new stem cell technologies
- \$150,000 direct project costs
- Project duration will be proposed and justified by applicant
- Applicant describes forward vision for a successful outcome
- High application volume expected

CIRM 2.0: Discovery Stage Programs:

PA 15-08: Quest Awards

- Goal is to discover a novel candidate therapeutic, device, diagnostic test or tool that can immediately progress to translation to enable broad use
- Project duration up to two years
- Direct project costs up to \$1.4M/award for a therapeutic candidate; or up to \$700,000/award for a candidate tool, device or diagnostic test
- Up to \$20,000/award to provide enrichment activities for graduate students, postdoctoral and clinical fellows on project
- Incentive offered for Progression Events – advancing candidate to translation
- High application volume expected

PA 15-08: **Quest Awards:** **Incentives for Progression**

- CIRM will offer an “Inception-like” Grant as a “reward” to Quest Award recipients who have achieved a “Progression Event” within one year of their Quest Award end date
- To achieve a Progression Event, Quest Awardee must submit evidence to CIRM indicating that additional funding has been acquired to support further development of the identified candidate
- CIRM Team will determine whether Progression Event has been met and at that time, verify that proposal for the grant reward is aligned with CIRM’s mission

CIRM 2.0: Discovery Stage Programs: PA 15-09: Challenge Awards

- Goal is to address Highly Specific Questions (HSQs) issued through periodic Challenge Question Announcements (CQAs)
- Research goals, level of support, award duration, and eligible activities will be tailored to a given HSQ and approved by President of CIRM
- Up to \$20,000/award to provide enrichment activities for graduate students, postdoctoral and clinical fellows on project
- Application volume expected to be low (5-10)

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CIRM 2.0: Discovery Stage Programs: Streamlined Review

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

- **Stage 1:** A pre-review will be conducted by 15 scientific GWG and 7 Patient Advocate members of GWG to select a subset of applications to advance to Stage 2 of review
- **Stage 2:** Standard, in person GWG review meeting resulting in funding recommendations to ICOC

This process improvement offers:

- Single step application process (No PreApps)
- Is scalable in response to application volume
- Can facilitate selection of proposal with high potential for impact from a broad applicant pool

CIRM 2.0: Discovery Stage Programs: Requested Decisions

- **Up to \$52.5 M for Discovery Stage Programs for Year 1:**
 - Up to \$44M for Inception, Quest, and Challenge Awards
 - Up to \$7.5M in reserve for funding Incentive Grants (rewards) for Quest Awardees who achieve Progression Events (“pass the baton”)
 - Up to \$0.7M for enrichment supplements to Quest and Challenge Award project graduate students, postdoctoral and clinical fellows
- **Requested delegation of board authority to President or his designee:**
 - To examine applications not selected for second stage of GWG review and make final determination whether to submit such to second stage or deny funding
 - To award Incentive Grant upon determination that Progression Event has been achieved and Incentive Grant proposal and budget is aligned with CIRM mission
 - To issue periodic Challenge Question Announcements that define a specific question and corresponding award terms under PA 15-09: Challenge Awards

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Translation Stage: 4 New Programs

PA 15-10:
Therapeutic Early
Development
Awards

PA 15-11:
Diagnostic Test
Early Development
Awards

PA 15-12:
Device Early
Development
Awards

PA 15-13:
Tool Early
Development
Awards

- Outcome links to CIRM clinical program

- Enrichment activities for students and fellows

- Outcome is readiness for clinical studies and/or final verification & validation studies for regulatory filing or for reference lab commercial use

- Enrichment activities for students and fellows

- Outcome links to CIRM clinical program or is readiness for final verification & validation studies for regulatory filing

- Enrichment activities for students and fellows

- Outcome is readiness for final verification & validation studies, for manufacturing & commercialization or readiness for broad use

- Enrichment activities for students and fellows

Translation Stage: Early Development Award Programs

- Therapeutics
 - Direct Project Costs up to:
 - \$5M for cell therapy candidate
 - \$2.5M for small molecule candidate
 - Project term up to 30 months and must be adequately justified
- Diagnostic Tests
 - Direct Project costs up to \$1.2M
 - Project term up to 24 months and must be adequately justified
- Devices
 - Direct Project costs up to \$2M
 - Project term up to 24 months and must be adequately justified
- Tools
 - Direct Project costs up to \$1M
 - Project term up to 24 months and must be adequately justified

CIRM 2.0: Translation Stage Programs: Requested Decisions

- **Up to \$40.5 M for Translation Stage Programs for Year 1:**
 - Up to \$40M for Therapeutic, Diagnostic Test, Device and Tool Early Development Awards
 - Up to \$0.5M for enrichment supplements to project graduate students, postdoctoral and clinical fellows
- **Requested delegation of board authority to President or his designee:**
 - To examine applications not selected for second stage of GWG review and make final determination whether to submit such to second stage or deny funding

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CIRM 2.0: Pathway to Patients

Discovery*, Translation* and Clinical Programs

Discovery

Translation

Clinical/Commercial

Progression Events

PA 15-07:
Inception Awards

PA 15-08:
Quest Awards

PA 15-09:
Challenge Awards

PA 15-10:
Therapeutic Early Dev.

PA 15-11:
Diagnostic Early Dev.

PA 15-12:
Device Early Dev.

PA 15-13:
Tool Early Dev.

PA 15-01:
Late Stage

PA 15-01:
Clinical

PA 15-01:
Accelerating

* Proposed

CIRM 2.0: Discovery & Translation Programs: Schedule and Deadlines for Year 1

Program	Cycles	Applications Due	Time to GWG	Time to Approval	Time to Launch
Discovery: Inception	annual	November	~ 3 mo. post submission	~ 5-6 mo. post submission	120 days post approval
Discovery: Quest	biannual	February, July	~ 3 mo. post submission	~ 5-6 mo. post submission	120 days post approval
Discovery: Challenge	biannual (as required)	February, July	~ 3 mo. post submission	~ 5-6 mo. post submission	120 days post approval
Translation: Early Development	biannual	March, September	~ 3 mo. post submission	~ 5-6 mo. post submission	90 days post approval

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Bridges 2.0: A Reimagined Initiative Better Serving CIRM's Mission

Objective: To prepare California's undergraduate and Master's level students for highly productive careers in stem cell research & therapy development.

What We Are Keeping

- Hands-on research internship
- Stem cell techniques training course
- Educational enhancement related to stem cells
- Broadening participation from under-represented populations



Bridges 2.0: A Revamped Initiative Better Aligned with CIRM's Mission

What We Are Adding

- Direct patient engagement activities
- Formal training on the regulatory pathway and development process
- Community outreach activities engaging diverse California communities in supporting healthcare initiatives
- Dynamic academic and career counseling

Bridges 2.0: A Revamped Initiative Better Aligned with CIRM's Mission

Who may apply?

- California universities or colleges that do not have a major stem cell research program

How will funds be awarded?

- Applications considered in a single review cycle

What is the requested funding allocation, project funding and project term?

- CIRM requests up to \$45.7 million to support up to 15 awards
- Each award will support up to 10 trainees per year
- Programs will be supported for up to 5 years

SPARK: Summer Program to Accelerate Regenerative Medicine Knowledge

Objective: To inspire, introduce and prepare California's high school students in the field of regenerative medicine as it relates to human diseases

What We Are Keeping from Creativity Awards:

- Full time hands-on summer research internship
- Broadening participation from under represented populations
- Poster Day



SPARK: Summer Program to Accelerate Regenerative Medicine Knowledge

What We Are Adding

- Direct patient engagement activities
- Preparatory training course before the commencement of research
- Community outreach activities engaging diverse California communities via social media

SPARK: Summer Program to Accelerate Regenerative Medicine Knowledge

Who may apply?

- California universities, colleges or research institutes with an established high school summer internship program

How will funds be awarded?

- Applications considered in a single review cycle

What is the requested funding allocation, project funding and project term?

- CIRM requests up to \$4 million to support up to 10 awards
- Each award will support a minimum of 5 and up to 10 trainees per year
- Programs will be supported for up to 5 years