Beyond CIRM 2.0

CALIFORNIA'S STEM CELL AGENCY

now it's personal

C. Randal Mills, Ph.D.
President and Chief Executive Officer

MISSION

CIRM's Mission

Accelerate stem cell treatments to patients with unmet medical needs.

The CIRM Spectrum

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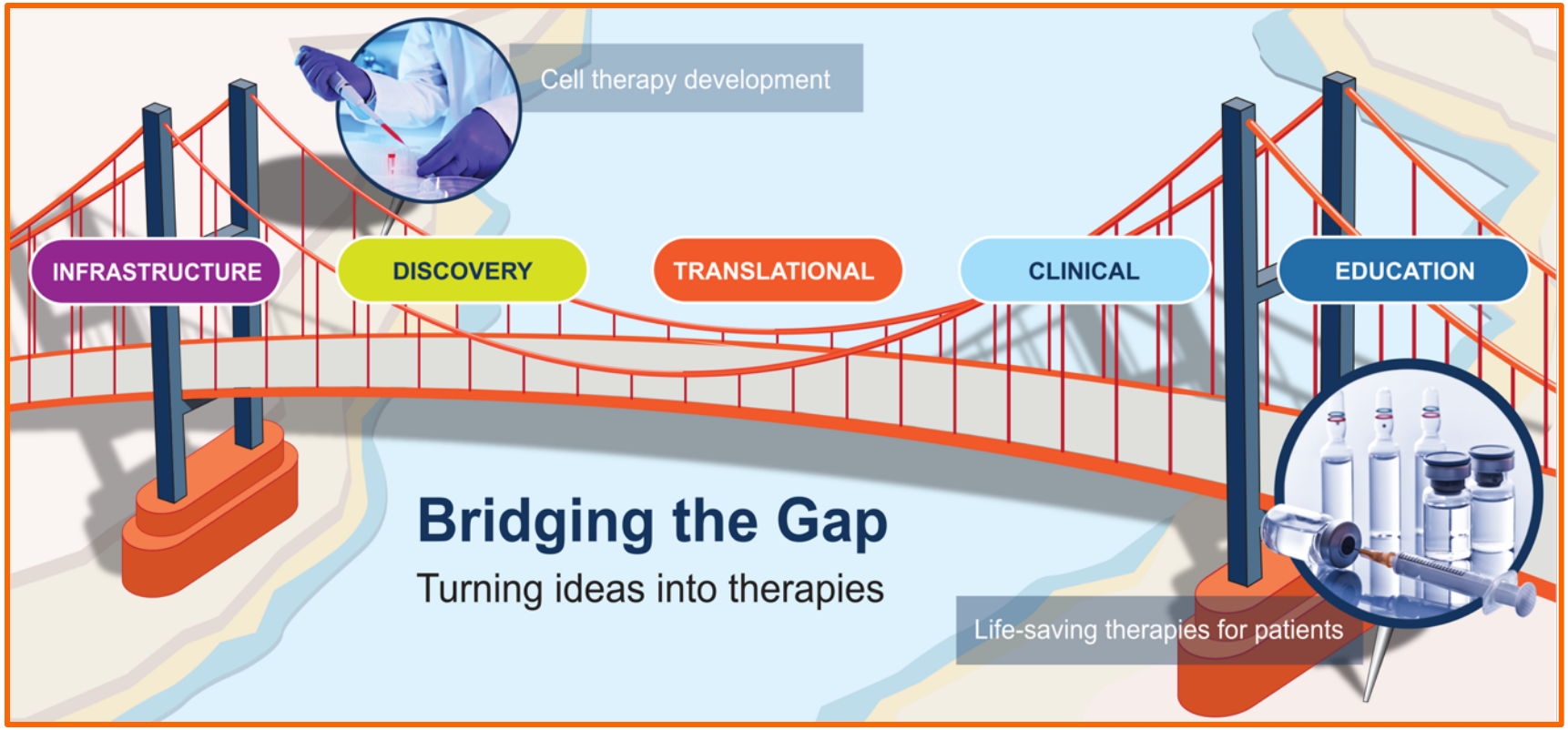
Five basic pillars of investment



Maximizing CIRM's Impact

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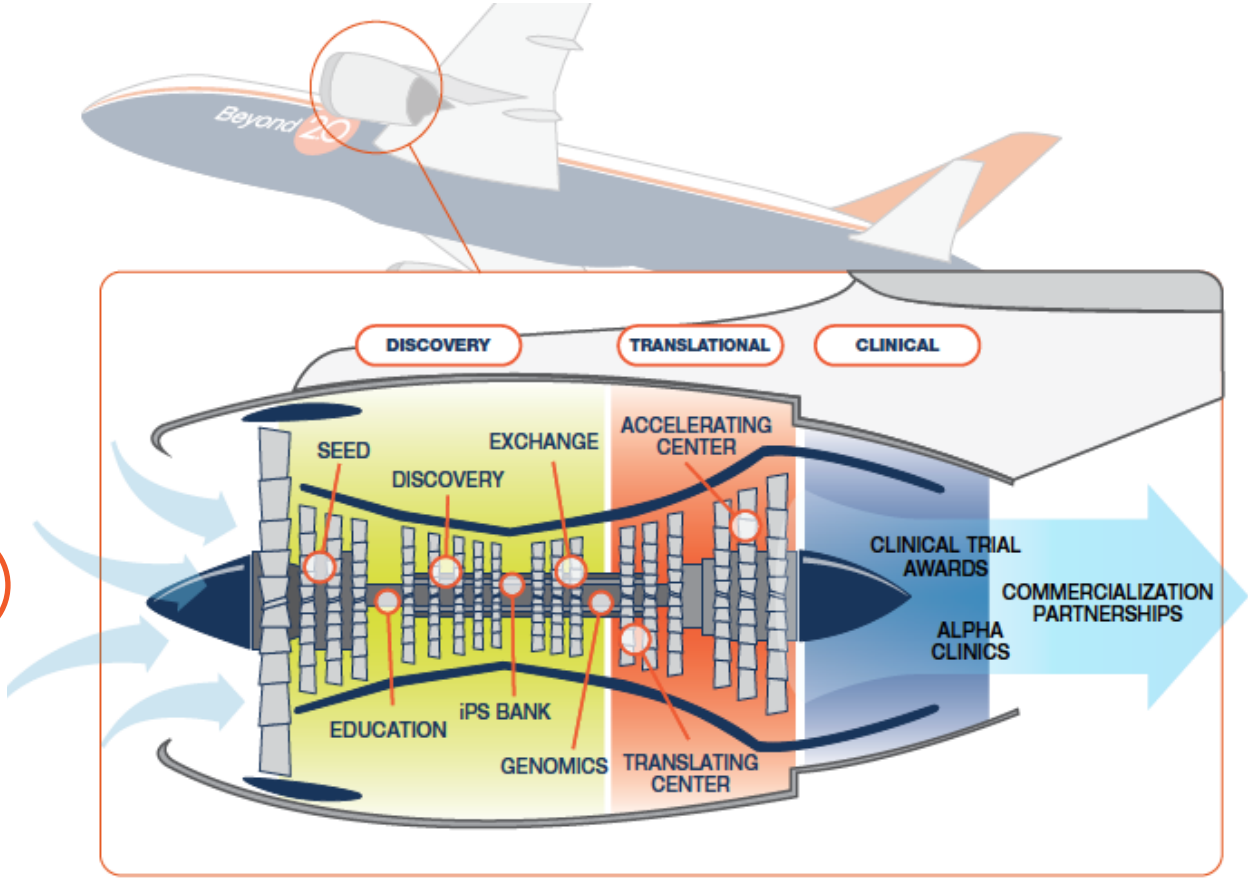
With approximately $800 million in new awards to be issued by the end of 2020, CIRM is still highly relevant

The amount of **new** awards CIRM estimates it will issue through 2020

Accelerating Success

With all of the pieces working together, CIRM is a stem cell accelerating machine

Ideas



Cures

Volume • Speed • Quality

A Seamless Pathway

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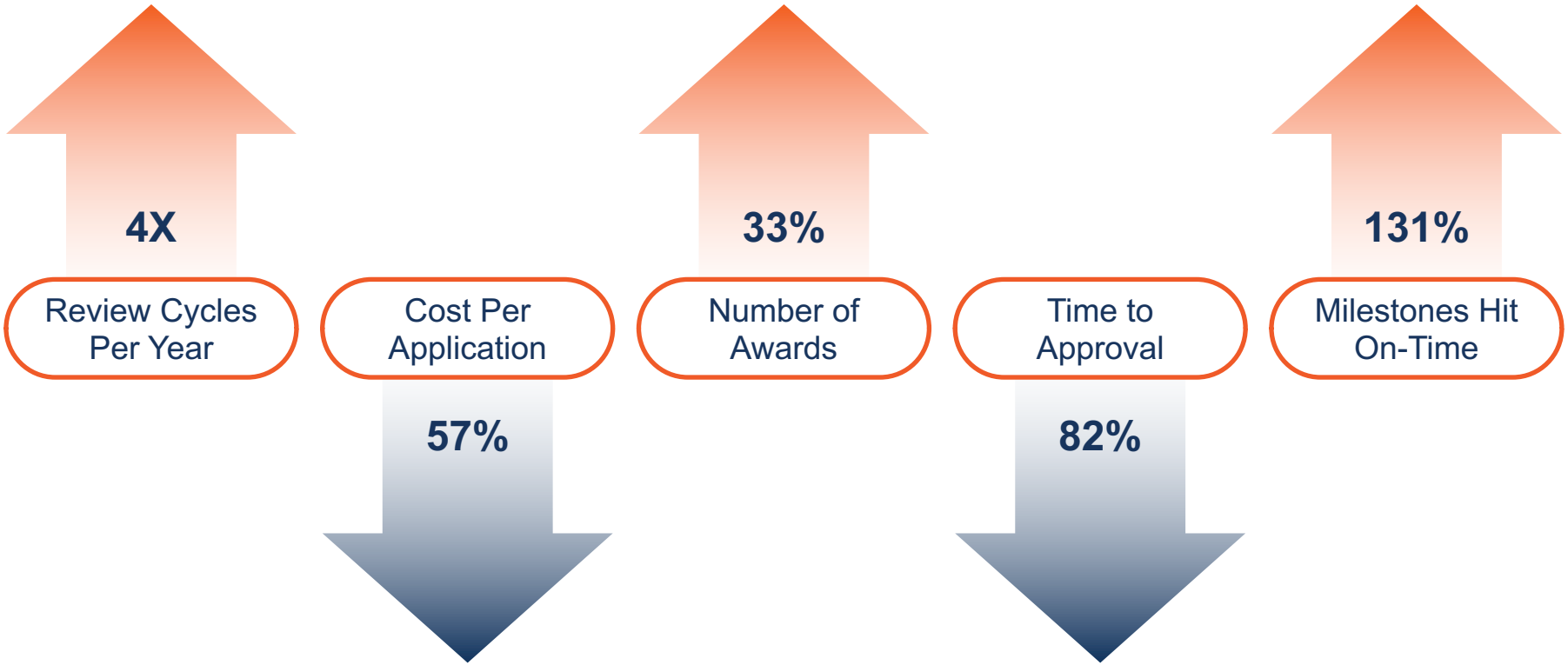
Program Offerings Per Year

CIRM 2.0 Performance

Volume, speed, quality and productivity are improving



Volume • Speed • Quality

Up Next!

Understanding the CIRM 2.0 process from start to finish

- Discovery & Translation stage programs – Pat Olson
- Clinical stage programs – Maria Millan
- How your grant is reviewed – Gil Sambrano
- Launching and administering your award – Gabe Thompson
- Legal considerations before you apply – Scott Tocher
- How CIRM's infrastructure can help you succeed – Neil Littman

CIRM ROADSHOW

Funding Early Stage Work

Pat Olson, Ph.D.

Vice President, Translation & Discovery

Funding Opportunities

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Program Offerings Per Year

Funding Opportunities

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DISCOVERY

New Idea → Single Product Candidate

Availability
2/Year

25-35 Awards Per Year

DISCOVERY 1 INCEPTION

Very early stage "seed" awards to generate preliminary data for potentially transformational ideas

Amount: $150k
Duration: Proposed by applicant
Prerequisite: None
Next: DISCOVERY 2

DISCOVERY 2 - QUEST

To promote the discovery of new stem cell-based therapeutics, diagnostics, devices or tool candidates that could ultimately enable broad use and improve patient care

Amount: $700k - 1.4M
Duration: 24 months
Opportunity for progression award
Prerequisite: None
Next: TRANSLATION 1-4

DISCOVERY 3 - CHALLENGE

To answer highly specific mission critical questions periodically posed by CIRM

Amount: Specified in RFA
Duration: Specified in RFA
Prerequisite: None
Next: Varies

Funding Opportunities

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TRANSLATION

Single Product Candidate → Pre-IND Meeting or Equivalent

Availability **3/Year**

10-15 Awards Per Year

TRANSLATION 1 THERAPEUTIC
For the development of a therapeutic candidate

Amount: Approximately $5M
Duration: 30 months

Prerequisite: Candidate
Next: CLINICAL 1

TRANSLATION 2 DIAGNOSTIC
For the development of a diagnostic device

Amount: Approximately $1.2M
Duration: 30 months

Prerequisite: Candidate
Next: CLINICAL 1 if applicable

TRANSLATION 3 DEVICE
For the development of a medical device

Amount: Approximately $2M
Duration: 24 months

Prerequisite: Candidate
Next: CLINICAL 1

TRANSLATION 4 TOOL
For the development of a stem cell tool

Amount: Approximately $1M
Duration: 24 months

Prerequisite: Candidate
Next: Commercialize of broad use

Discovery & Translation

Keys to ensuring a successful CIRM partnership

- Focus and commitment
- Timely, open communication and reporting
- Forward thinking

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Funding Clinical Trials

Maria Millan, M.D.

Vice President, Therapeutics

Funding Opportunities

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Program Offerings Per Year

Funding Opportunities

Pre-IND Meeting or Equivalent → Approved Therapy

Availability
12/Year

All Meritorious Applications

CLINICAL 1 IND ENABLING

For the development of candidates from the Pre-IND meeting stage to filing a successful IND

Amount: $5M
Target 18 months

Prerequisite: Pre-IND Meeting
Next: CLINICAL 2

CLINICAL 2 CLINICAL TRIAL

For the conduct of any phase of a clinical trial

Amount: Up to $20M
Duration: 60 months maximum

Prerequisite: Active IND
Next: CLINICAL 2
As needed

CLINICAL 3 ACCELERATING ACTIVITY

Supplemental funding for opportunistic activities that will accelerate the overall development of an active Clinical 1 or Clinical 2 candidate

Amount: As needed
Duration: As needed

Prerequisite: CLINICAL 1 or CLINICAL 2
Next: None

Scope of Clinical Grants

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Clinical stage grants cover Pre-IND through all stages of clinical trials

- Stem Cell or Progenitor Cell therapeutic candidates
- Hematopoietic Stem Cells being developed in a novel way to address a rare condition or unmet medical need
- Small molecules or biologics that target stem cells and are not likely to be funded from other sources

Co-Funding Requirements

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Matching funds vary by phase of trial and type of institution

Applicant Type	Pre-Clinical	Phase 1	Phase 2	Phase 3	CLIN3
Non-Profit	None	None	40%	50%	Same as parent award
For-Profit	20%	30%	40%	50%	Same as parent award

Therapeutics

Keys to ensuring a successful CIRM partnership

- Engage the CIRM Therapeutics team to help with the Application
- Present a Clear and Compelling “Value Proposition”
- Strong data and development plan

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Who is Eligible and How to Apply

Gil Sambrano, Ph.D.

Director, Portfolio Development & Review

What is the process for review?

Process Time: 80 to 105 days

Essential Reading

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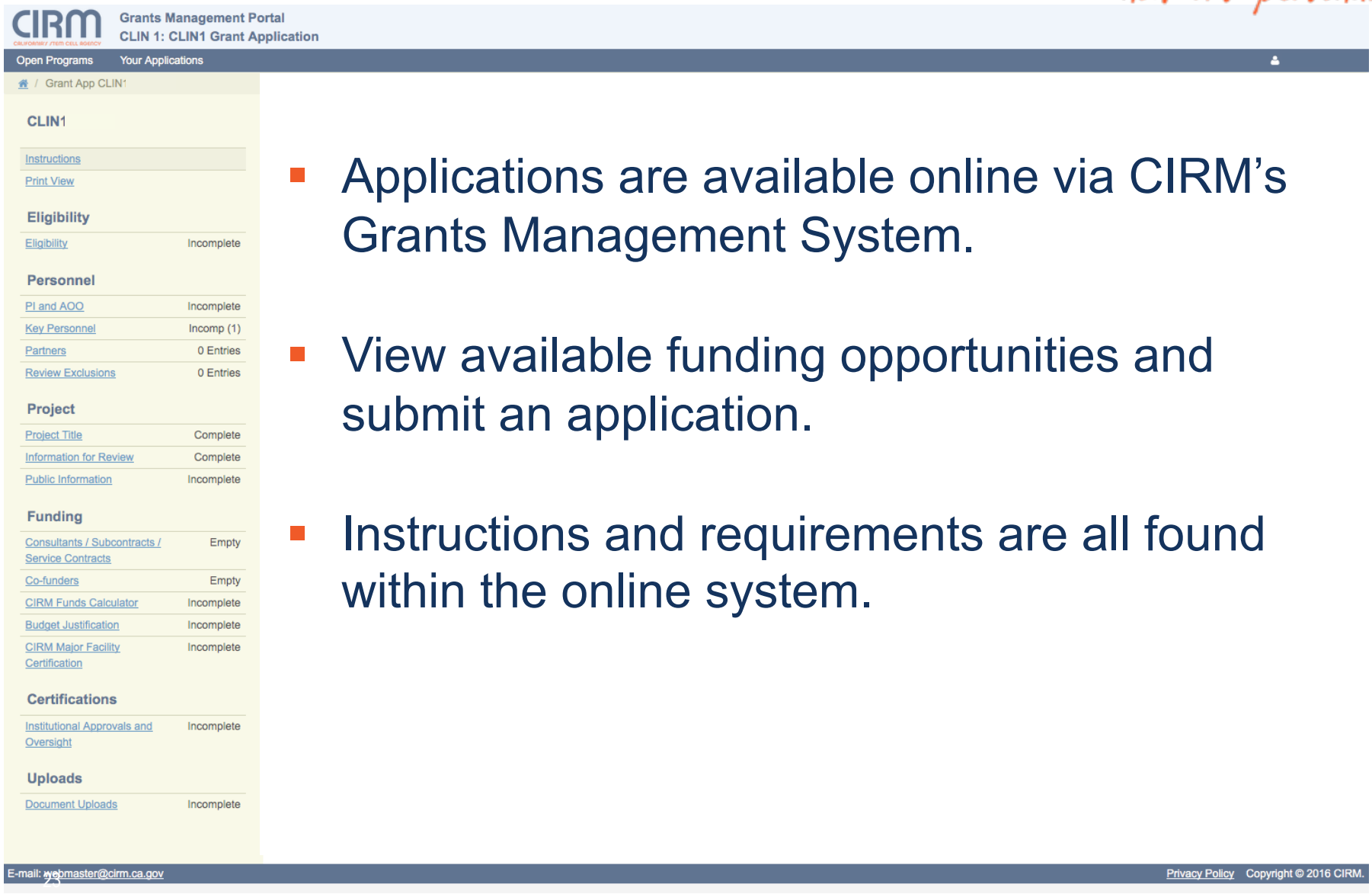
What is CIRM looking for and how do I partner with CIRM to achieve the mission?

How do you apply?



- Applications are available online via CIRM's Grants Management System.
- View available funding opportunities and submit an application.
- Instructions and requirements are all found within the online system.

What does CIRM check for?

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- ✓ Complete and accurate
- ✓ Solvency (if a company): 180 days cash-on-hand
- ✓ Co-funding requirement: availability of funds
- ✓ Proposed therapeutic qualifies as a stem cell-based therapy
- ✓ Minimum regulatory approvals are in place (e.g., IND)
- ✓ Status as a California based organization
- ✓ Budget is reasonable for proposed activities
- ✓ Contingency Plan

= Go to next stage of review

Who are the GWG?

- The Grants Working Group (GWG) is responsible for the scientific review of all applications.
- The GWG is composed of 23 members:
  - 7 patient advocate members of CIRM's Governing Board
  - 15 scientists external to California
  - Chair of the Board (ex-officio)
- The GWG makes funding recommendations to CIRM's Board.

What does the GWG look for?

- ✓ Does the project hold the necessary significance and potential for impact?
- ✓ Is the rationale sound?
- ✓ Is the project well planned and designed?
- ✓ Is the project feasible?

What do GWG scores mean?

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Clinical Applications

(Scored 1, 2 or 3)

Disc/Tran Applications

(Scored 1 - 100)

3
Do Not Fund*

2
Needs Improvement**

1
Warrants Funding

<85
Do Not Fund

85+
Warrants Funding

*Imposes a six months deferral before reapplication
**Responses to question and any amendments can be submitted immediately

What happens at the CIRM Board?

- Meetings are public
- Occur monthly, mostly by teleconference
- Presentation of GWG recommendations
- Includes scores and summary of key strengths/weakness
- Vote to fund or not to fund

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What Happens After You are Approved for Funding

Gabriel Thompson

Director, Portfolio Operations and Performance

CIRM's Board Approved Your Application… **Now What?**

- **Launch:** CIRM's Just-in-Time (JIT) process for award contracting activities.
- **Milestones & Disbursements:** Now that we have the award, what do we need to do to continue to get paid?
- **Reporting:** Tell us how it's going!

Award Launch

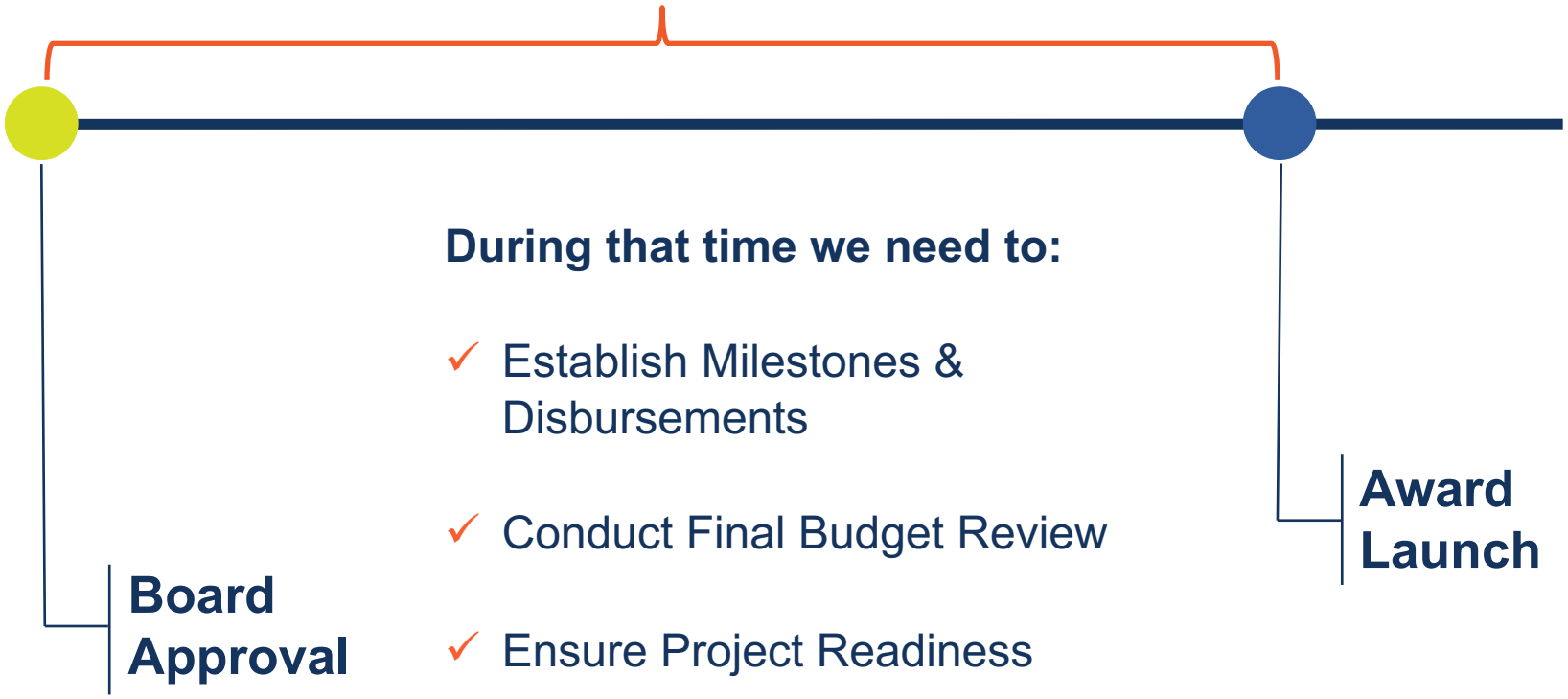
Moving from Board approval to funding

Targets

Clinical Stage Programs = **45 days**

Translational Stage Programs = **90 days**

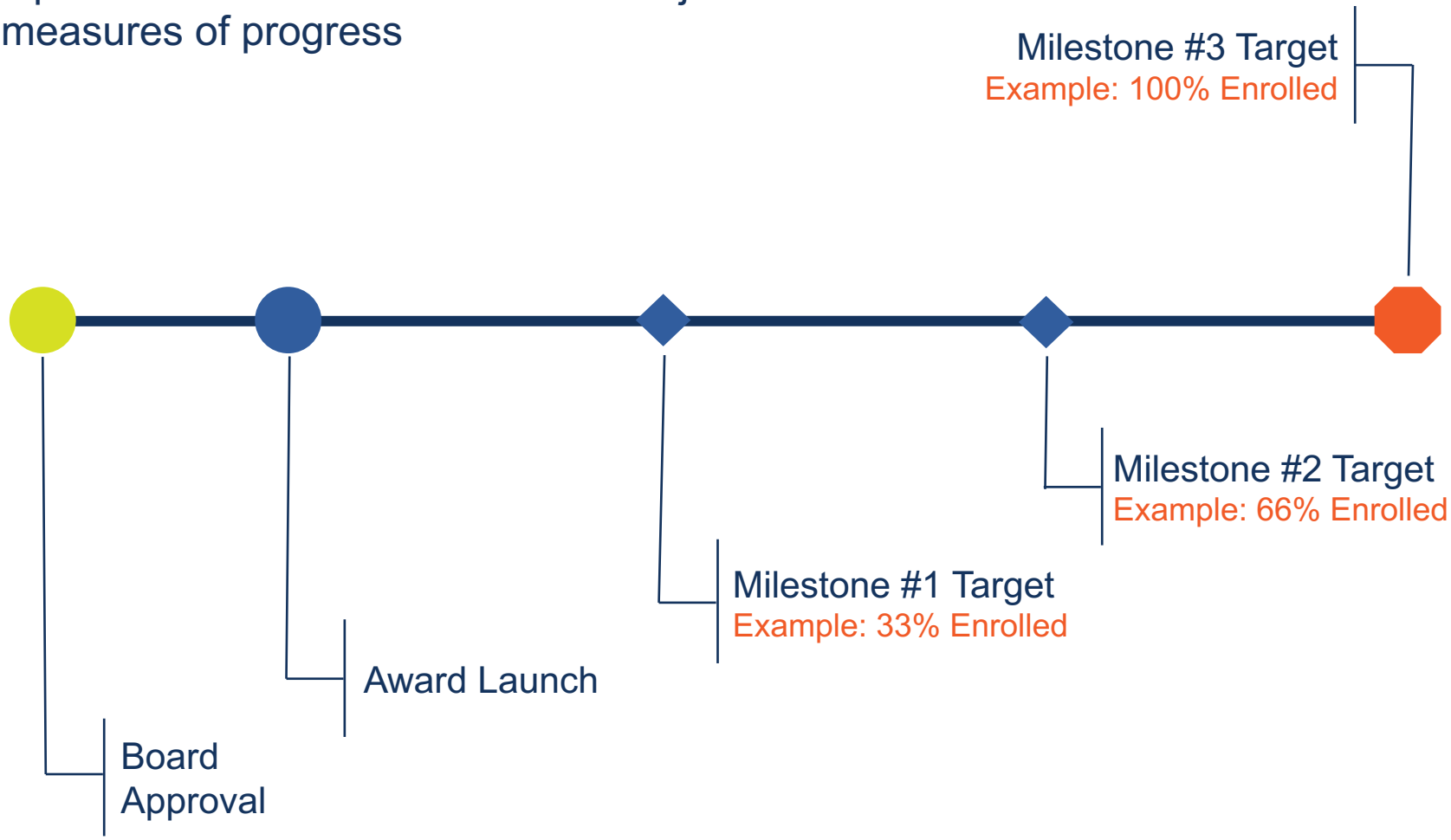
Discovery Stage Programs = **120 days**



Milestones & Disbursements

Example for a $3M Award

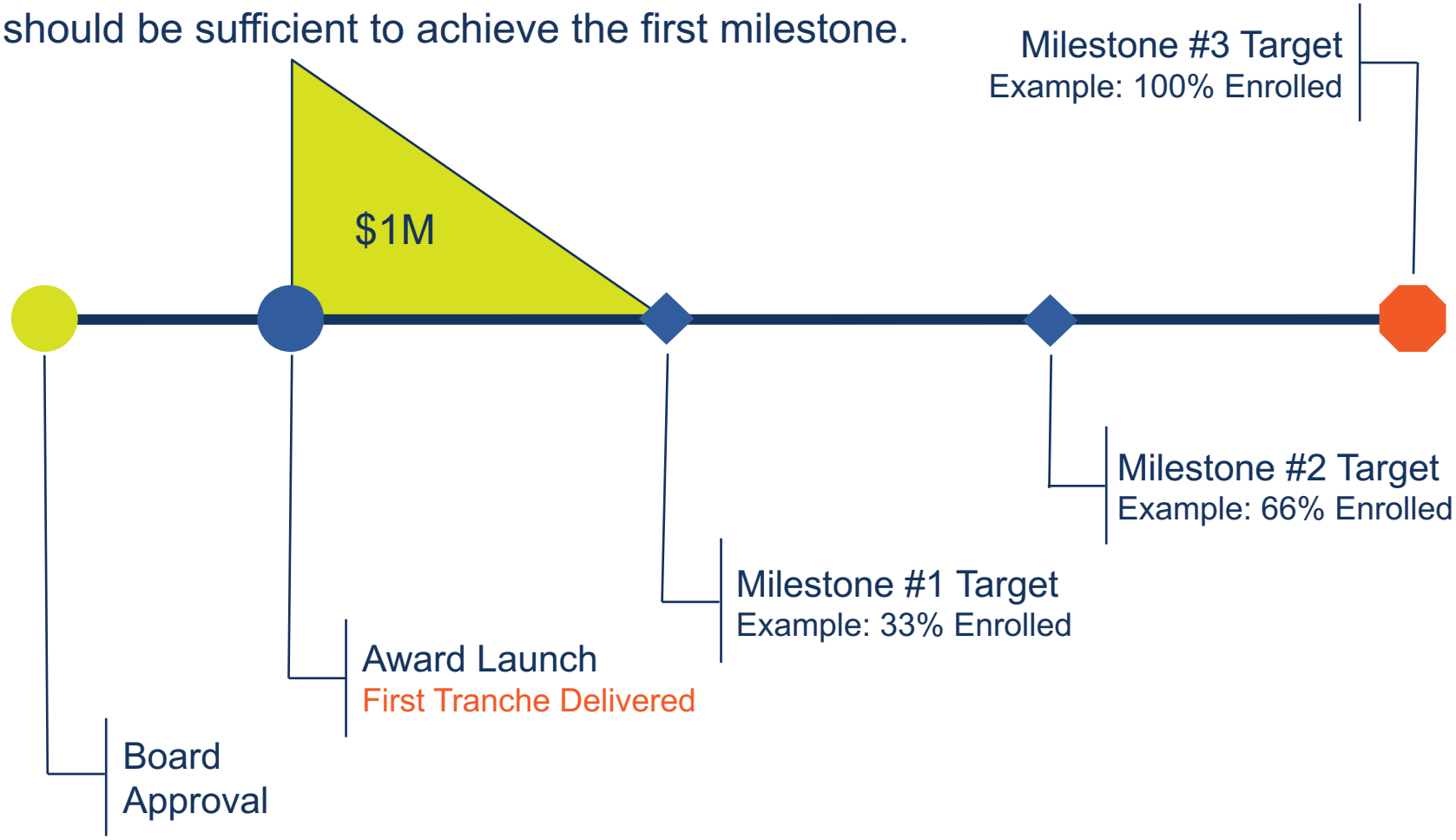
Operational milestones are set on objective measures of progress



Milestones & Disbursements

Example for a $3M Award

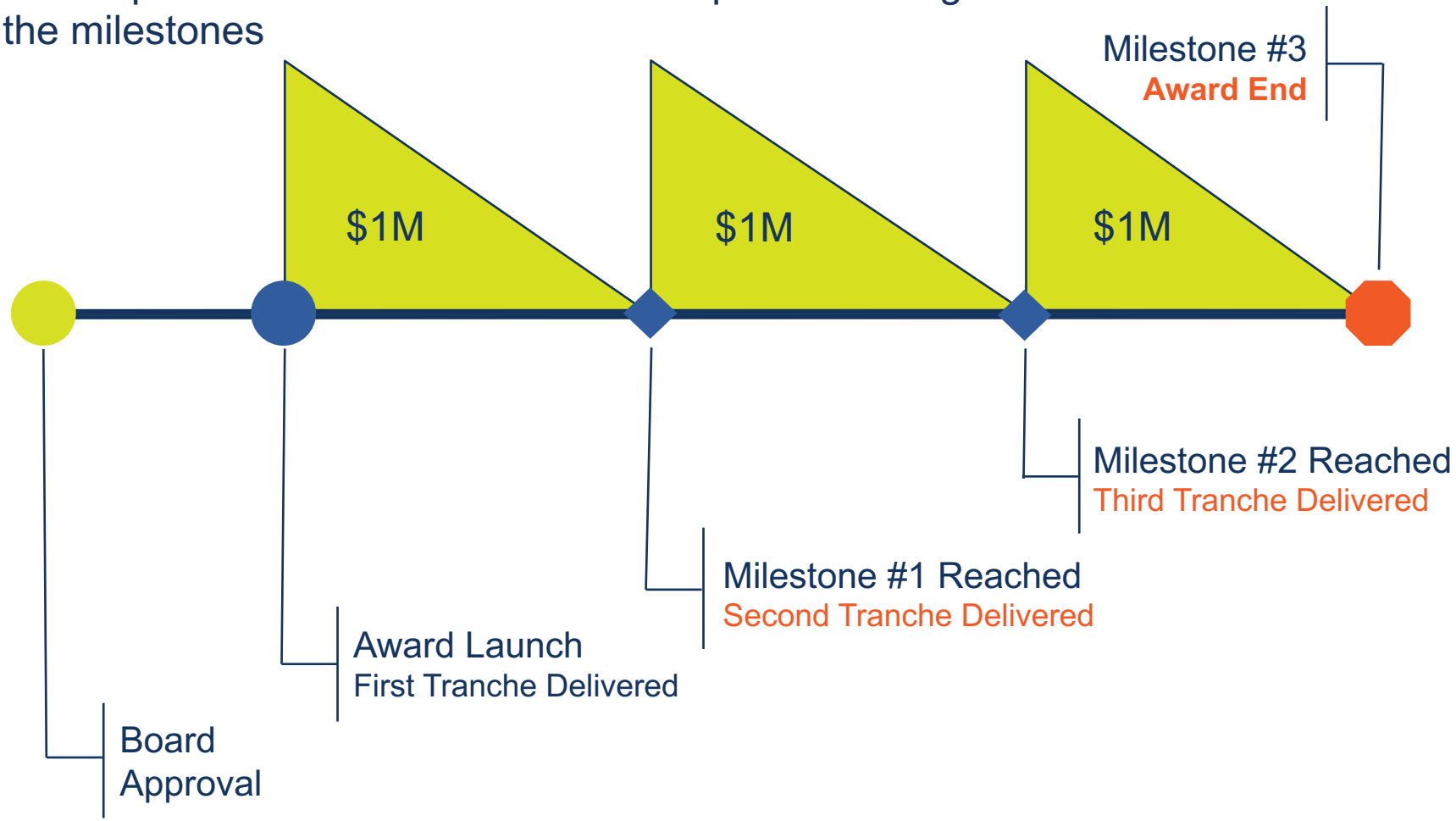
The first disbursement is made at award launch and should be sufficient to achieve the first milestone.



Milestones & Disbursements

Example for a $3M Award

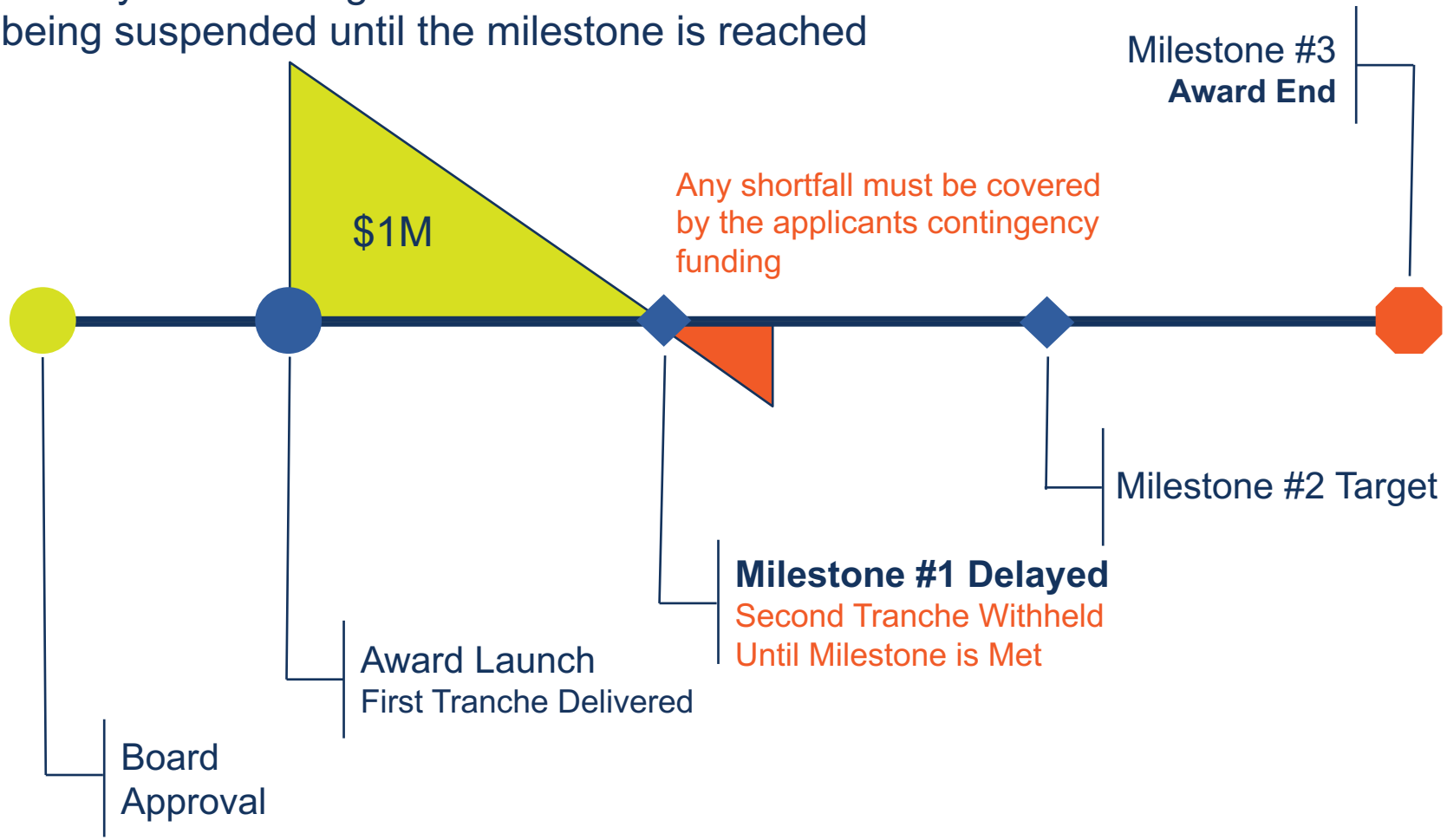
Subsequent disbursements are made upon achieving the milestones



Milestones & Disbursements

Example for a $3M Award

A delay in achieving a milestone results in disbursements being suspended until the milestone is reached



Milestones & Disbursements

Example for a $3M Award

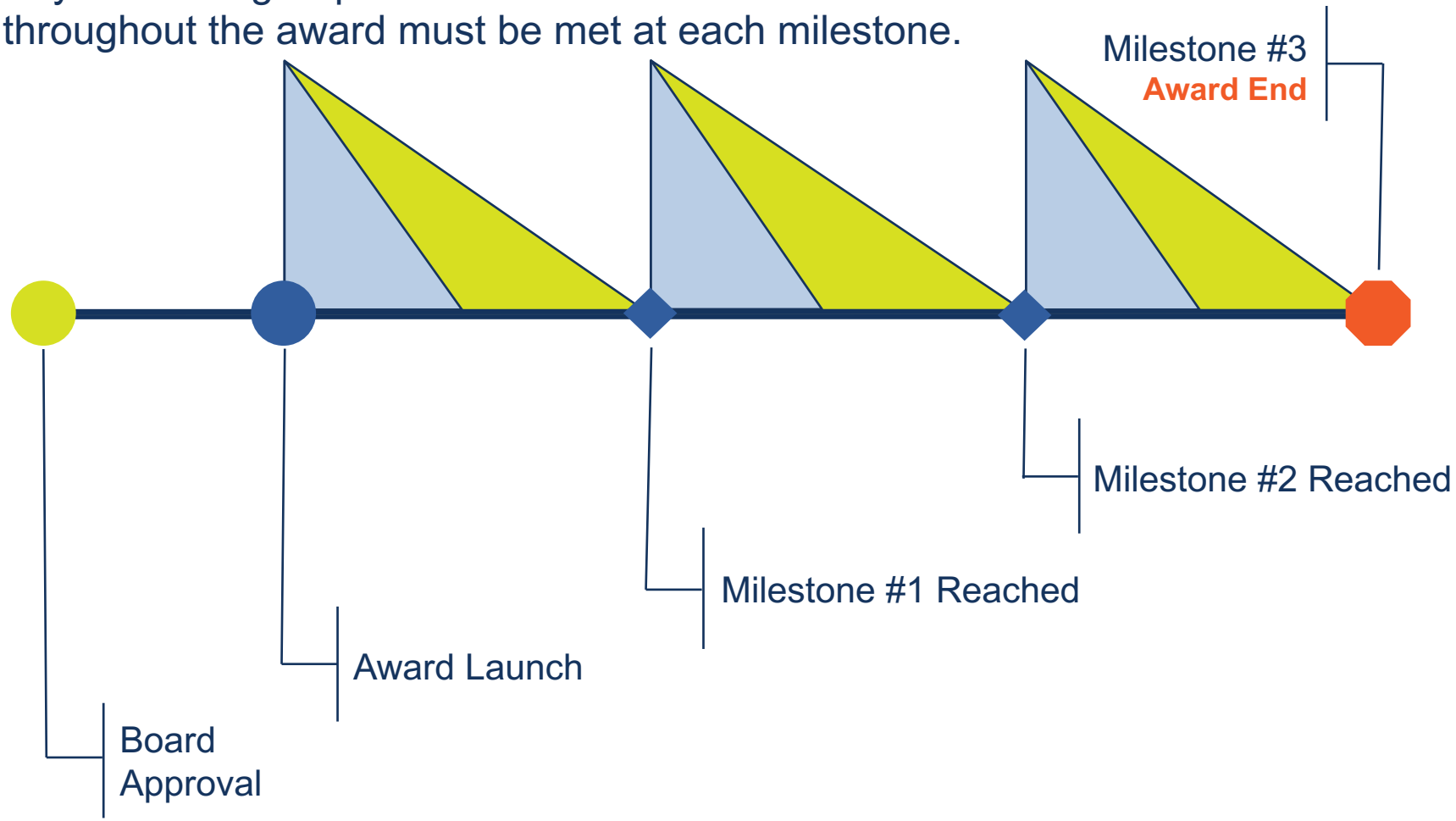
If a milestone is reached early, any savings is carried forward.

Any excess at **award end** can be used to further the project or similar research.

Co-Funding Requirements

Example for an award requiring 50% matching

Any co-funding requirement that is continuous throughout the award must be met at each milestone.



Award Monitoring

- **Progress and Financial Reports**
  - Milestone focused
  - Within scope

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Rules and Regulations governing CIRM Awards

Scott Tocher, Deputy General Counsel

Legal Support & Information

- What you need to know before applying

What Applicants Need to Know:

Make sure you meet the eligibility requirements

- CIRM Team cannot waive them

What Applicants Need to Know:

Make sure proposal describes the planned work

- Contracts are performance-based and Project Scope cannot be renegotiated after Board approval

What Applicants Need to Know:

Review CIRM policies – IP, Medical and Ethical Standards, and Grants Administration Policy

- Policies are State Law and CIRM Team cannot waive them

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How our Translating & Accelerating Centers and the Alpha Clinic Network can Help You Succeed

Neil Littman

Director, Business Development & Strategic Infrastructure

Strategic Infrastructure Programs

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CIRM
ALPHA STEM CELL CLINICS NETWORK
Accelerating Patient Treatments

CORIELL INSTITUTE
FOR MEDICAL RESEARCH

Stem Cell Accelerator Center | QUINTILES

Accelerating Therapies
a public private partnership
ATP3

CIRM
CALIFORNIA'S STEM CELL AGENCY
TRANSLATING CENTER

Alpha Stem Cell Clinics

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Currently supporting 23 clinical trials involving ~140 patients

Accelerating Center: Driving stem cell clinical development from IND to market

Stem Cell Accelerator Center | QUINTILES

Immediate benefits of engagement

- ✓ Prepare and optimize grant applications and budgets
- ✓ Prepare and file INDs
- ✓ Plan, design, and execute high quality clinical trials
- ✓ Preferred pricing to CIRM grantees

Open for business

Grand Opening Ceremony October 4th!

Translating Center: Preclinical Research Organization

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CIRM CALIFORNIA'S STEM CELL AGENCY TRANSLATING CENTER

- IND enabling regulatory studies
- Process development
- cGMP manufacturing
- Consultation services

Launching 4Q-2016

Accelerating Therapies:
Public-Private Partnership (ATP3)

Up to a **$75 million** investment to advance CIRM technology

For more information:

Understanding the CIRM 2.0 process

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