

CURE

Beyond
CIRM 2.0
CALIFORNIA'S STEM CELL AGENCY

now it's personal



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

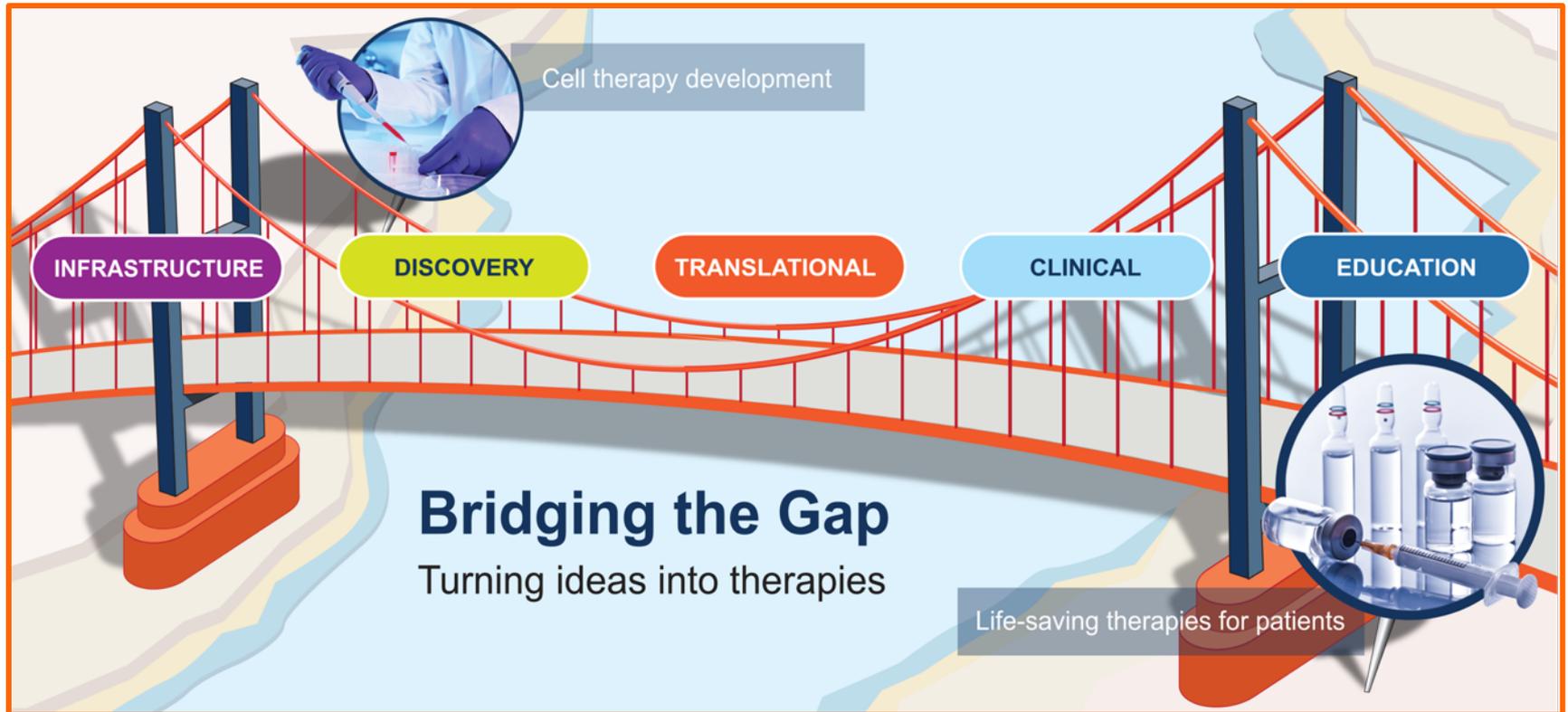
HOPE

CIRM's Mission

Accelerate stem cell treatments to patients with unmet medical needs.

The CIRM Spectrum

Five basic pillars of investment



Maximizing CIRM's Impact

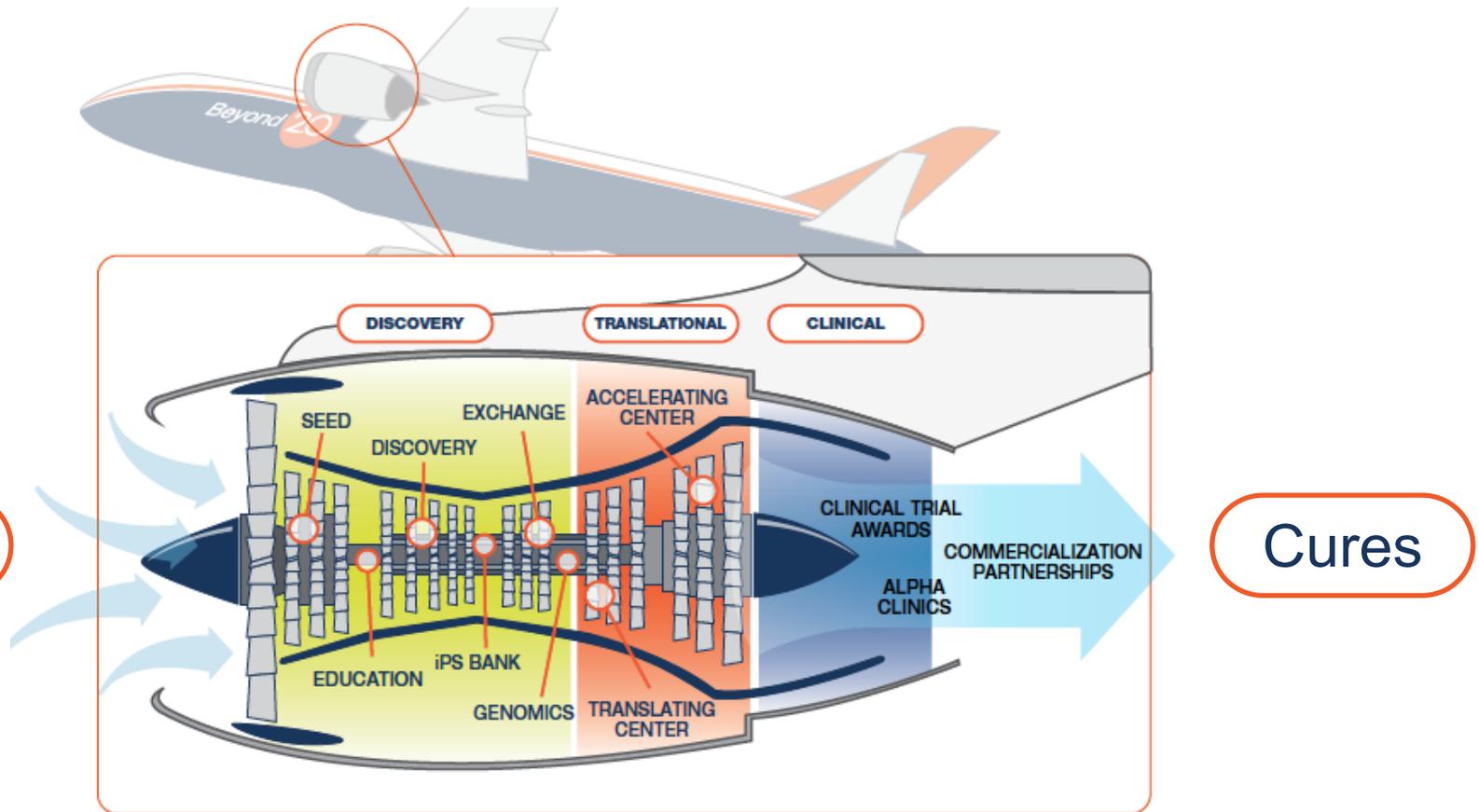
With approximately \$800 million in new awards to be issued by the end of 2020, CIRM is still highly relevant



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



Volume • Speed • Quality

A Seamless Pathway



DISCOVERY



TRANSLATION



CLINICAL

New Idea



Single Product Candidate



Pre-IND Meeting or Equivalent



Approved Therapy



2/Year



3/Year

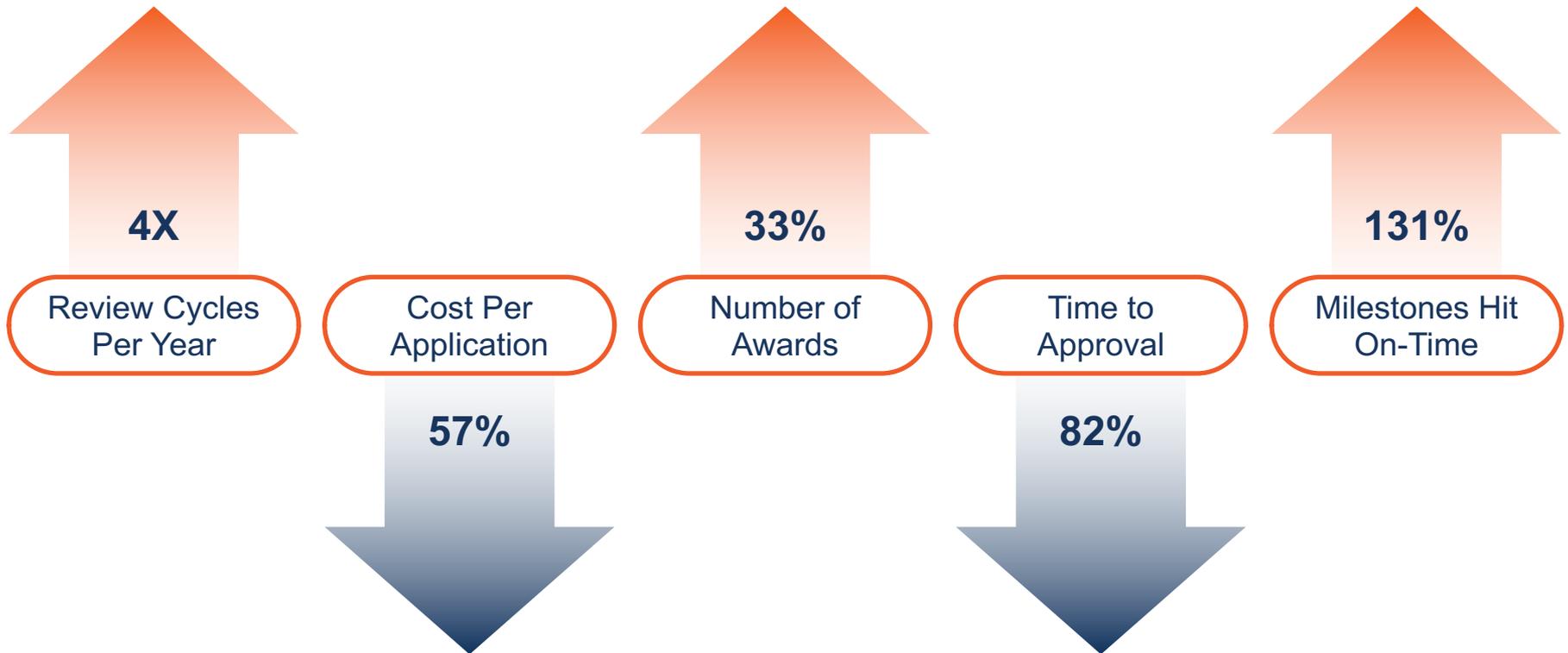


12/Year

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



DISCOVERY



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12/Year

Program Offerings Per Year

Funding Opportunities



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



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



DISCOVERY



TRANSLATION



CLINICAL

New Idea



Single Product Candidate



Pre-IND Meeting or Equivalent



Approved Therapy



2/Year



3/Year



12/Year

Program Offerings Per Year

Funding Opportunities



CLINICAL

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

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

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

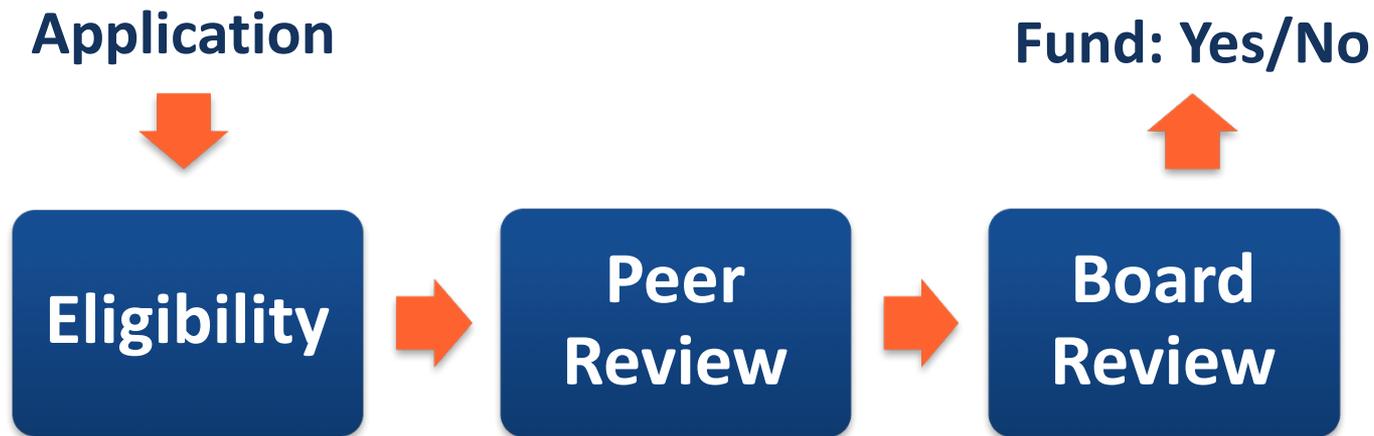
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Who is Eligible and How to Apply

Gil Sambrano, Ph.D.
Director, Portfolio
Development & Review

What is the process for review?



Process Time: 80 to 105 days

Essential Reading



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

How do you apply?

CLIN1

Instructions

[Print View](#)

Eligibility

[Eligibility](#) Incomplete

Personnel

[PI and AOO](#) Incomplete

[Key Personnel](#) Incomp (1)

[Partners](#) 0 Entries

[Review Exclusions](#) 0 Entries

Project

[Project Title](#) Complete

[Information for Review](#) Complete

[Public Information](#) Incomplete

Funding

[Consultants / Subcontracts / Service Contracts](#) Empty

[Co-funders](#) Empty

[CIRM Funds Calculator](#) Incomplete

[Budget Justification](#) Incomplete

[CIRM Major Facility Certification](#) Incomplete

Certifications

[Institutional Approvals and Oversight](#) Incomplete

Uploads

[Document Uploads](#) Incomplete

- 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?

- ✓ 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

PASSED

= 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?

Clinical Applications

(Scored 1, 2 or 3)

Disc/Tran Applications

(Scored 1 - 100)

3

Do Not Fund*

<85

Do Not Fund

2

Needs Improvement**

1

Warrants Funding

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

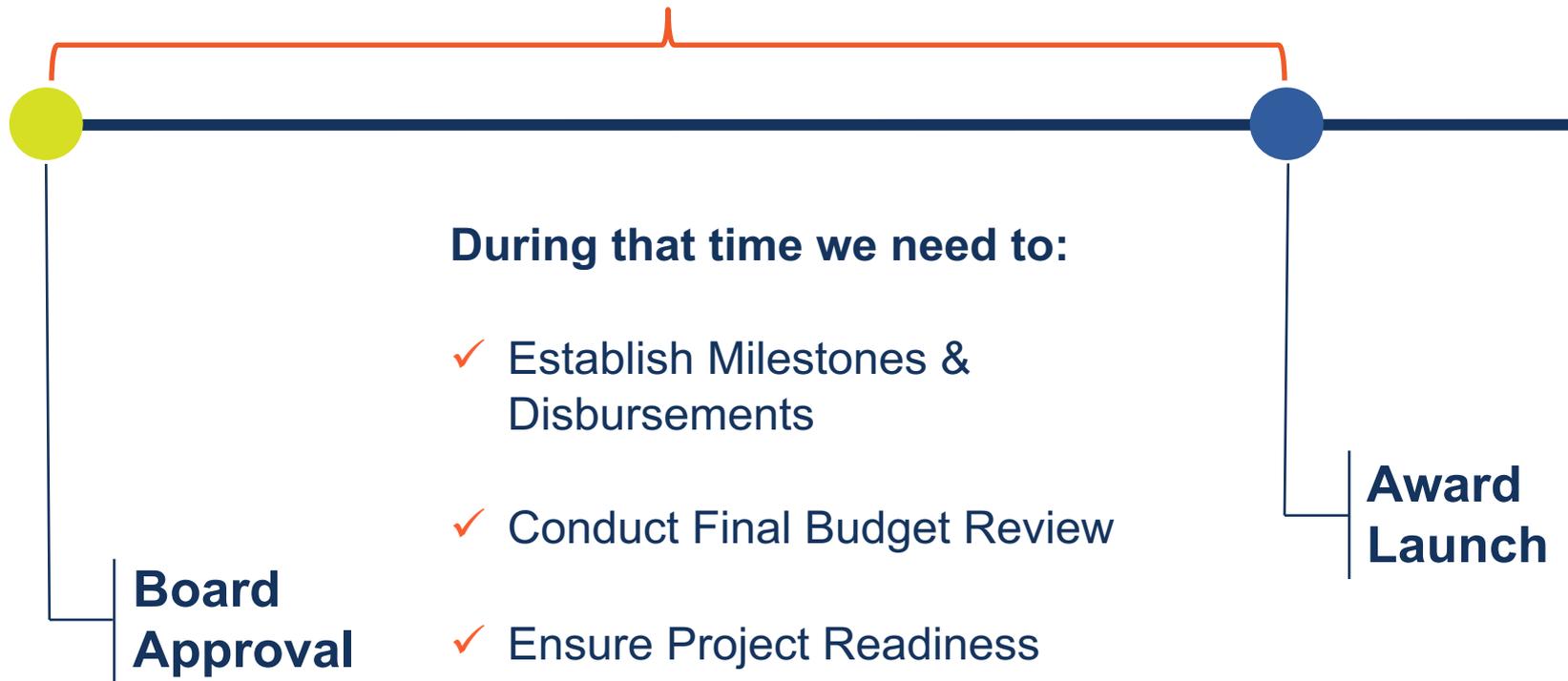
Moving from Board approval to funding

Targets

Clinical Stage Programs = **45 days**

Translational Stage Programs = **90 days**

Discovery Stage Programs = **120 days**



Milestones & Disbursements

Example for a \$3M Award

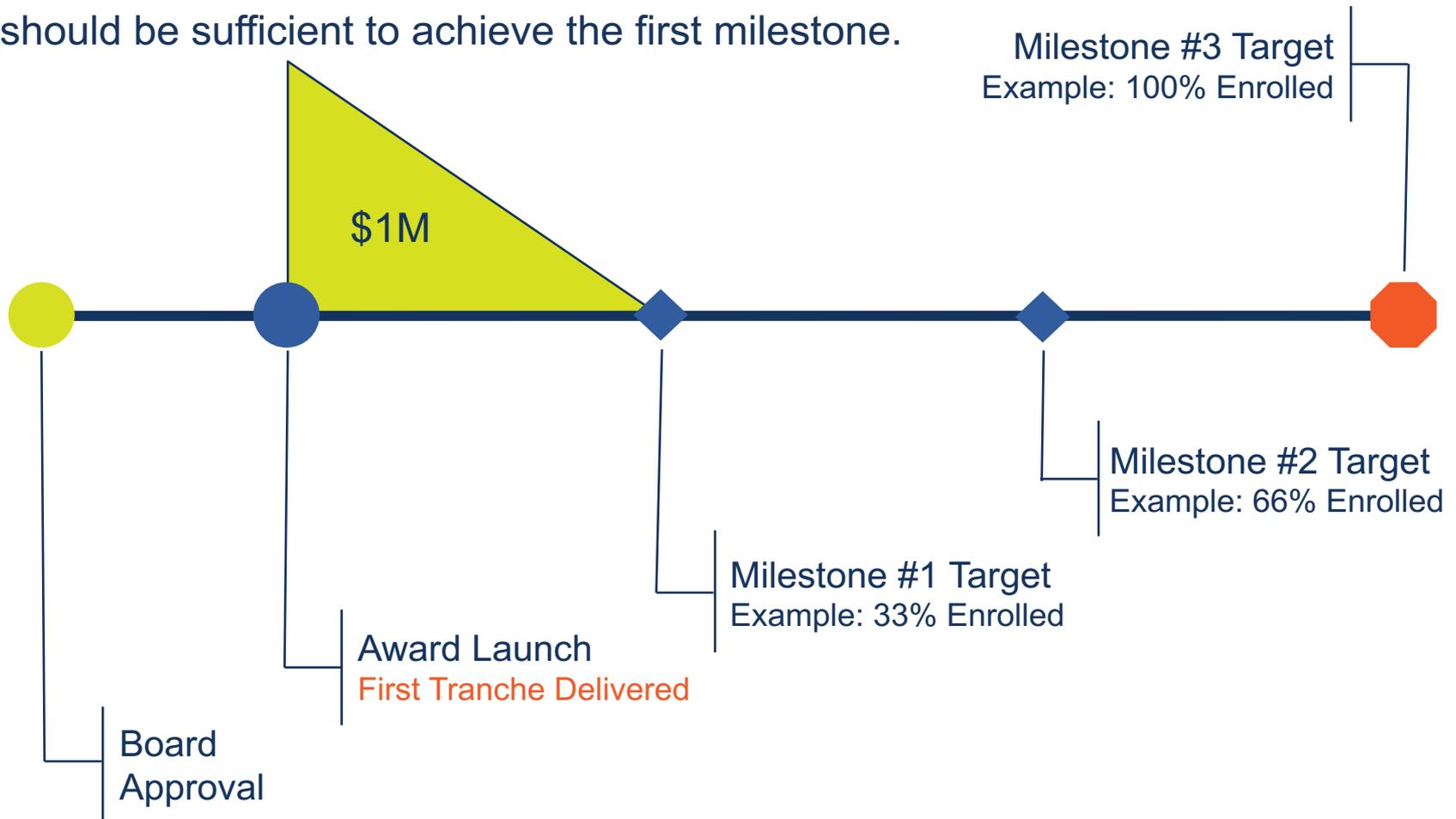
Operational milestones are set on objective measures of progress



Milestones & Disbursements

Example for a \$3M Award

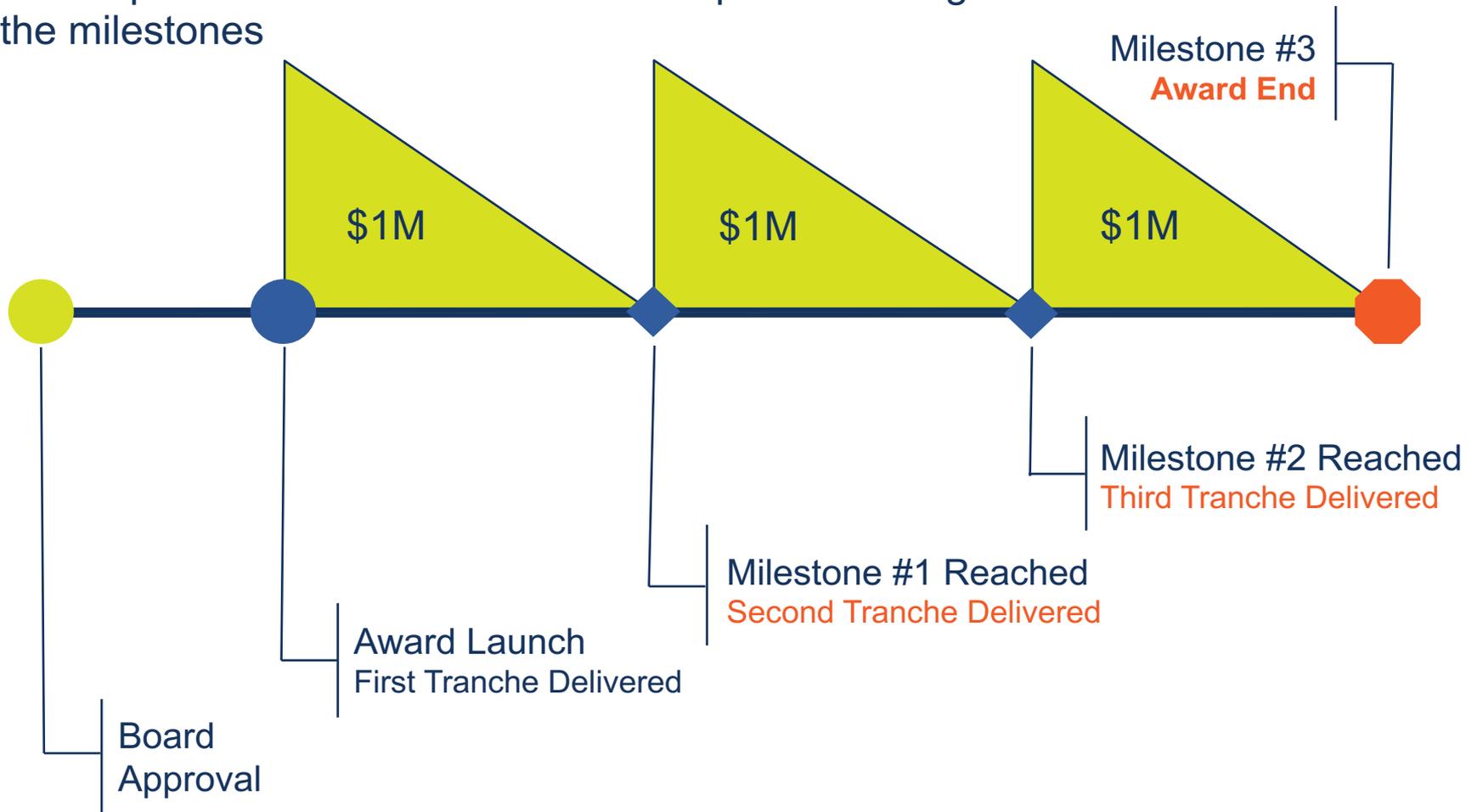
The first disbursement is made at award launch and should be sufficient to achieve the first milestone.



Milestones & Disbursements

Example for a \$3M Award

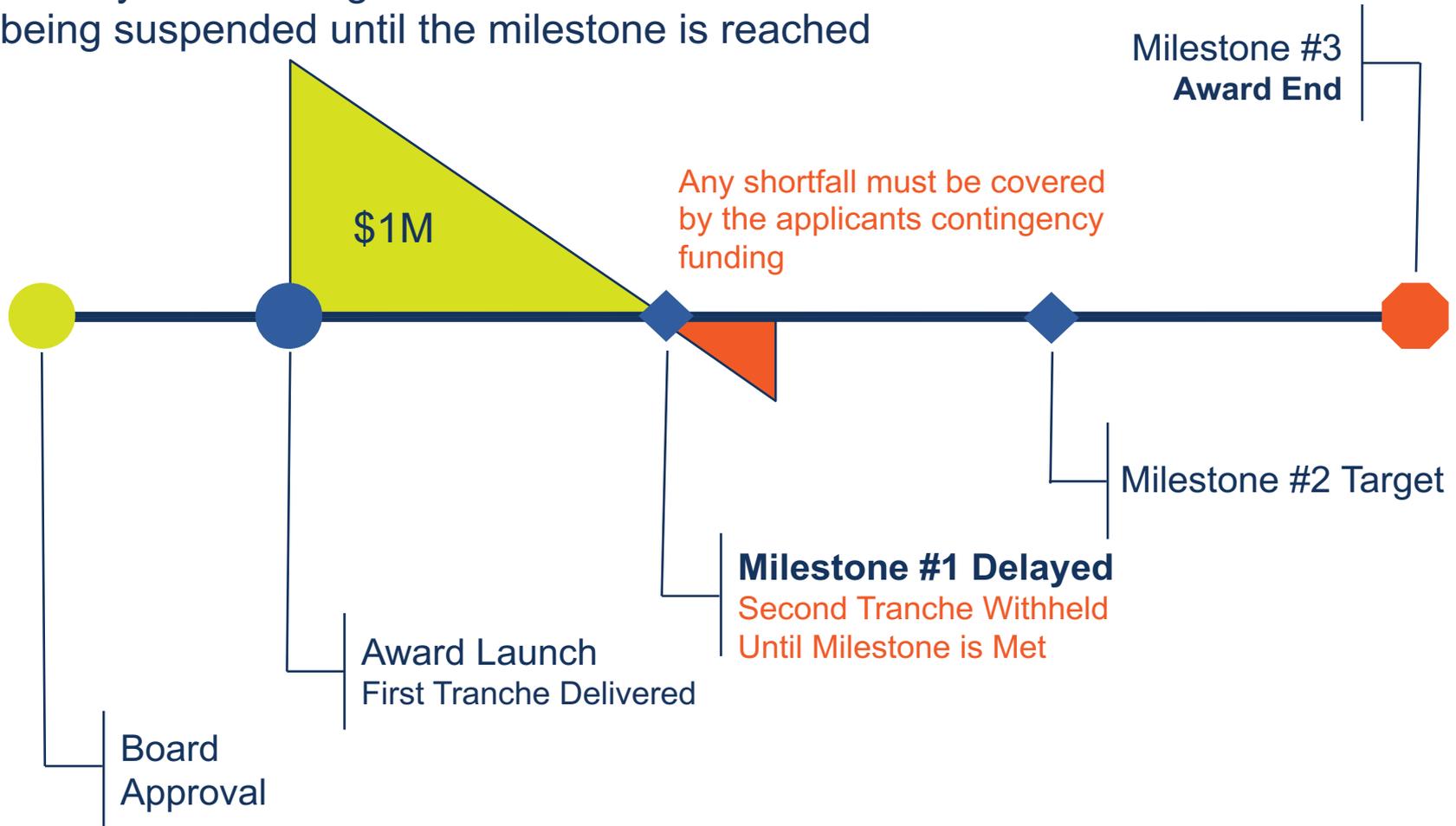
Subsequent disbursements are made upon achieving the milestones



Milestones & Disbursements

Example for a \$3M Award

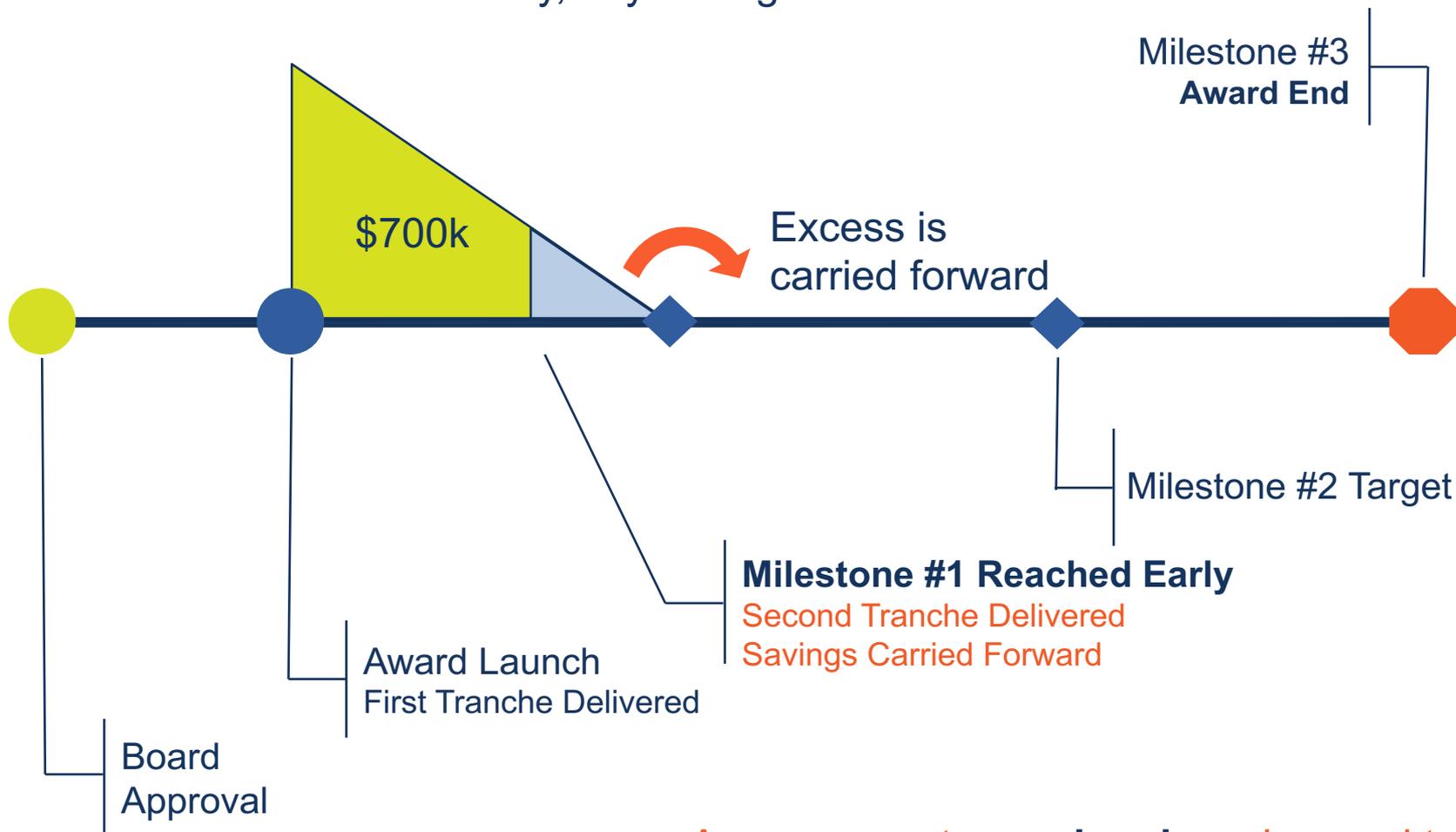
A delay in achieving a milestone results in disbursements being suspended until the milestone is reached



Milestones & Disbursements

Example for a \$3M Award

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

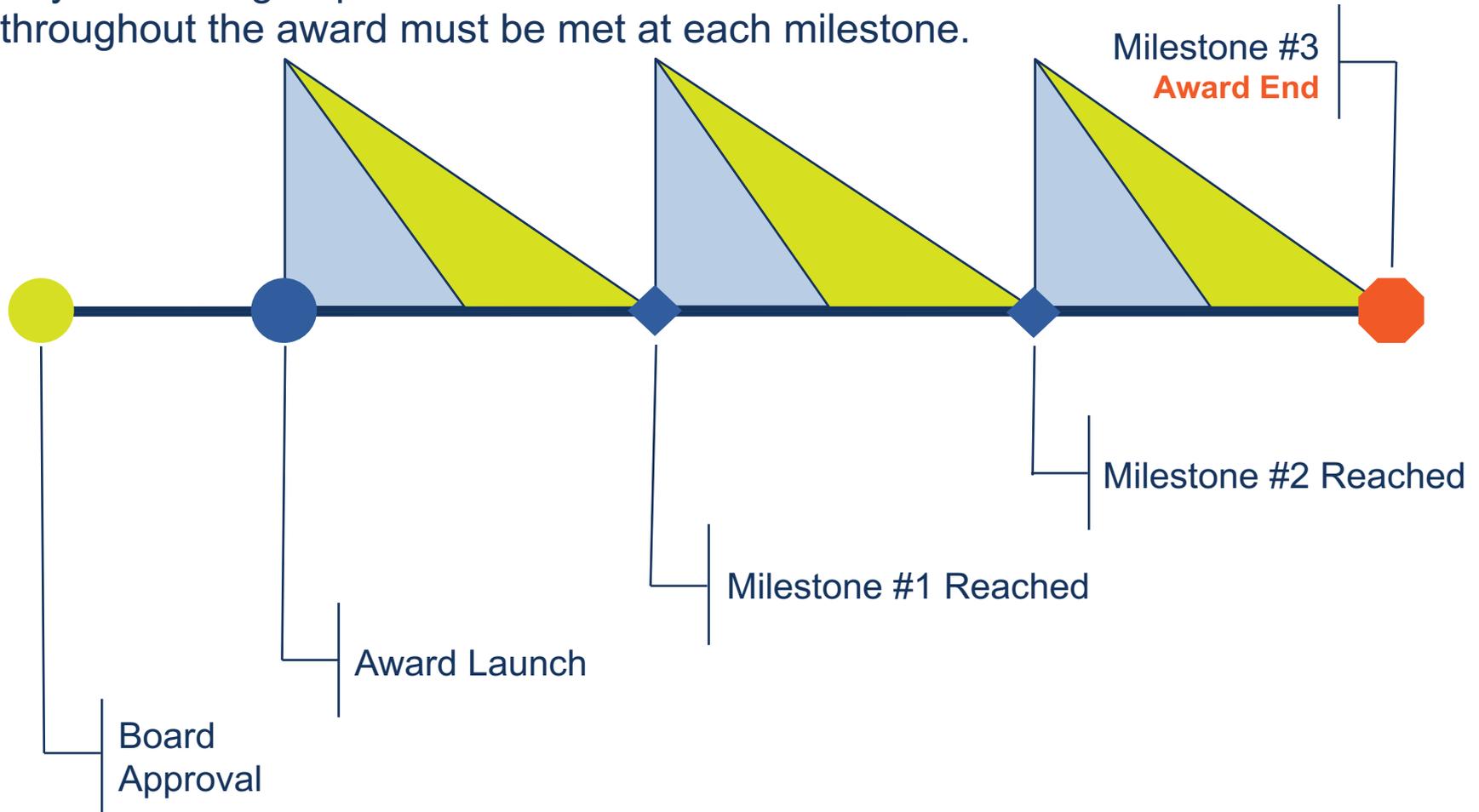


Any excess at **award end** can be used to further the project or similar research.

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
- Allowable costs
- Converting Grants to Loans

What Companies Need to Know



Make sure you meet the eligibility requirements

- CIRM Team cannot waive them

What Companies Need to Know



Make sure your proposal describes the planned work

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

What Companies Need to Know



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

- Policies are State Law and CIRM Team cannot waive them

What Companies Need to Know



Two policies may be of particular interest

- Allowable cost rules
- Grant-to-loan conversion option

Allowable Project Costs



Different Rules for California (CA) and Non-California (Non-CA) Organizations:

- CA Organization = $>50\%$ CA employees
- Non-CA Organization = $\leq 50\%$ CA employees

For A California Organization

Best Deal!

**Allowable
Costs**

=

Research Conducted in
California

+

Non-California Research
Conducted Under the Control
of the Awardee

Non-CA Allowable Project Costs



Clinical Research = Portion of the fully loaded clinical trial cost as determined by the percentage of patients enrolled in California

Example: Total enrollment = 100 patients

California Patients = 25

Fully Loaded Trial Cost = \$10M

Allowable costs = 25% or \$2.5M

Grant-to-Loan Conversion Option

For Clinical & Translational Projects

Award Type	Royalty	Repayment	Pricing Access March-In
Loan	X	✓	✓
Grant	✓	X	✓

Awardee can convert a grant to a loan the earlier of 10 years from date of award or 10 days after FDA marketing application with immediate repayment.

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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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now it's personal



CORIELL INSTITUTE
FOR MEDICAL RESEARCH

Stem Cell
Accelerator Center



Accelerating Therapies **ATP³**
a public private partnership



CIRM **TRANSLATING CENTER**
CALIFORNIA'S STEM CELL AGENCY

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



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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- 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

- Discovery & Translation stage programs – Pat Olson
polson@cirm.ca.gov
- Clinical stage programs – Maria Millan
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- How your grant is reviewed – Gil Sambrano
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