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12/14/23 ICOC Meeting





#### **OUR MISSION**

Accelerating world class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world



# Proposed DISC0 and DISC2 Concept Amendments

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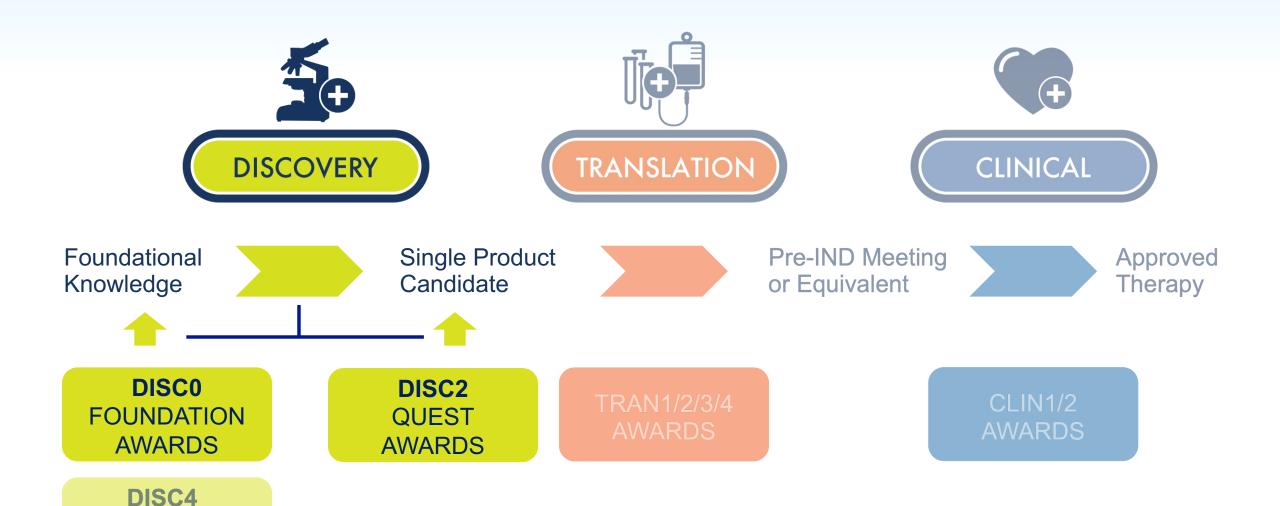




ReMIND

**AWARDS** 

# **DISCOVERY Pillar Programs**





# CIRM Main Changes to DISC2 Concept

- 1. Award Tracks
- 2. Award Budgets



## **DISC2 Awards: Changes in Track**

#### **Current Tracks**

- Therapeutic Candidate
- Diagnostic/Tool/Device Candidates

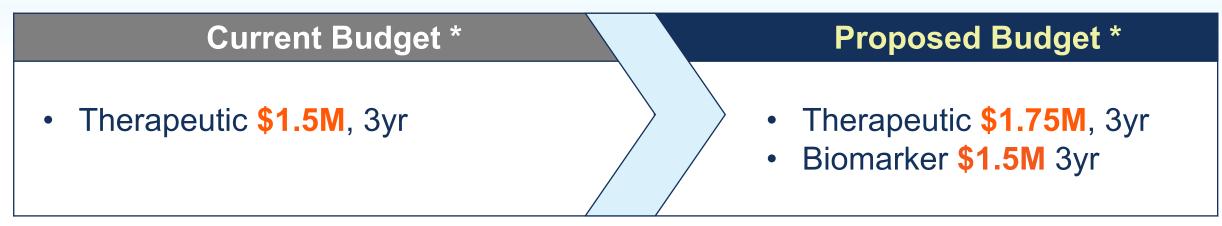
#### **Proposed Tracks**

- Therapeutic Candidate
- Biomarker Candidate

- Tool/Device development and proof of concept is already supported by DISC0 mechanism
- Biomarker track: Includes Diagnostic + Expands scope to high unmet need in Tx Dev of Regenerative Medicine treatments (and CNS diseases)



## **DISC2 Awards: Changes in Budget**



<sup>\*</sup> Direct project costs for entire award

- Increased award accounts for higher costs of trainees/research
- \$200K special supplement expanded to all therapeutic applications via higher base budget
- Budget and duration for biomarker track comparable to similar awards

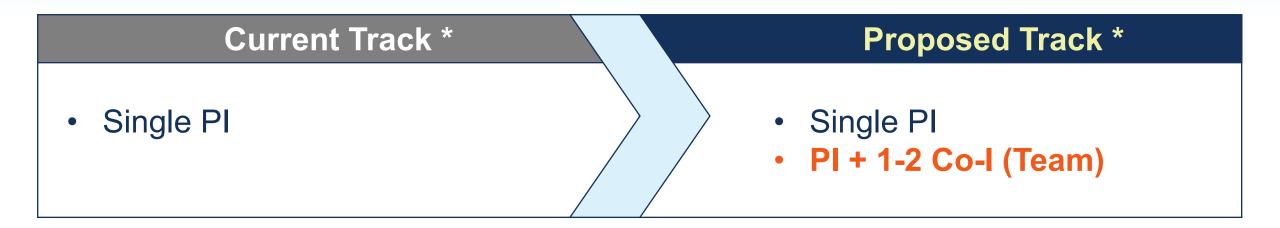


# CIRM Main Changes to DISC0 Concept

- 1. Award Tracks
- 2. Award Budgets
- 3. PI Percent Effort



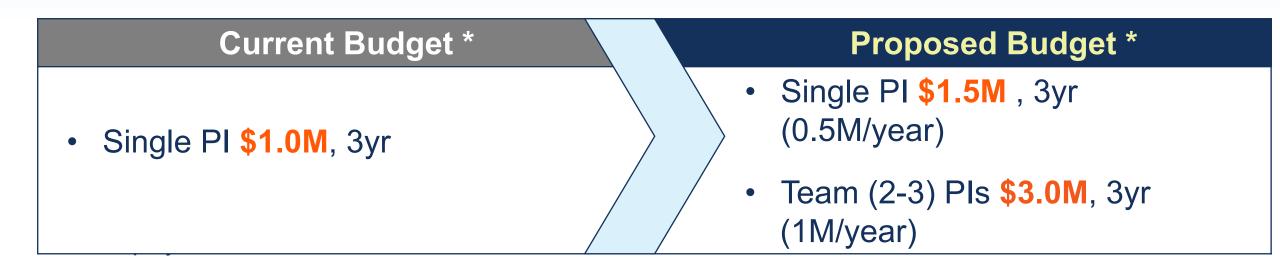
## **DISC0 Awards: Changes in Track**



- Team track will support synergistic, multidisciplinary collaborations with larger scope
- Teams drive innovation, creativity, and risk-taking with the support of a group



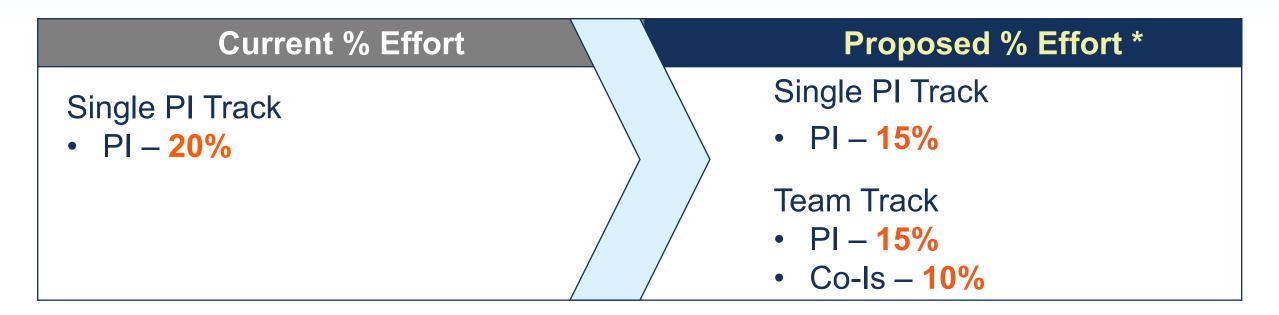
## **DISCO Awards: Changes in Budget**



- Budget increased to account for higher costs of trainees/research
- Budget and duration in line with similar awards for Team Track



## **DISC0 Awards: Changes in Percent Effort**



#### Rationale

Board feedback/alignment with other funding bodies



# CIRM requests ICOC approval of these amendments



# Proposed CLIN Concept Amendments and CLIN4 Concept Proposal

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## **Proposed CLIN Concept Changes**

## What is CIRM recommending?

- 1. Remove clinical track for medical devices
- 2. Increase maximum award amounts for CLIN1
- 3. Update CLIN2 PA to highlight specific allowable activities for product development
- 4. Introduce new CLIN4 PA to fund Late-Stage Development activities necessary to file a BLA and readiness for product launch



## Clinical Programs Awards Overview

+	Aim / Scope	Eligibility	Duration
CLIN 1	IND Enabling	Pre-IND Meeting / Start in 45 Days	24 Months*
CLIN 2	Clinical Trial	Active IND / Start in 45 Days	48 Months
CLIN 4	BLA Enabling	Active CLIN2 /End Of Phase 2 mtg	48 Months



<sup>\*</sup> Up to 6 months additional for start-up activities



## CIRM CLIN1 Awards: Changes in Award Amount

#### Maximum total CIRM award amount

Current Budget	Proposed Budget
<ul> <li>Non-profits, \$6 M</li> <li>For-profits, \$4 M</li> </ul>	<ul> <li>Non-profits, \$7.5 M</li> <li>For-profits, \$5 M</li> </ul>

- Increased award amounts accounts for toxicology studies, manufacturing, and higher workers wages costs
- Duration for CLIN 1 remains at 24 months



## Recommended CLIN2 Concept Changes

# Why are changes needed?

- When CLIN2 was implemented, most CIRM-funded trials were in Early-stage Clinical Development
  - Phase 1 or Phase 2
- As the field has matured, more programs are entering Late-stage Development
  - Phase 3 or Pivotal/registration trial stage directly from phase 1/2 dependent on the disease indication
- The current CLIN2 Program Announcement is not clear or explicit about the support of specific late-stage development activities
- To Ensure Best Practices Alignment with the FDA



### Clarification of Eligible Activities in CLIN2

#### **Activities** that qualify for funding under CLIN2:

- Compilation of alternative comparator data acceptable to FDA for a marketing approval decision and intended to support the proposed interventional clinical trial in cases where placebo or sham controls are not possible.
  - Examples include Natural History studies or use of existing data repositories
  - Must have documented FDA agreement on the acceptability of the proposed comparator
- Compilation of Patient-Reported Outcomes (PRO) related to the conduct of the proposed trial
- Compilation of Real -World Data (RWD) and Real- World Evidence (RWE) related to the conduct of the proposed trial
- Activities that support **DEI** goals described in the proposal



#### What is the purpose of a CLIN4?

- Certain key activities required by FDA to get to Biologics License Application (BLA) filing and readiness for product Launch are not covered by the current CLIN2 PA
- The goal of a CLIN4 is to support CIRM-funded programs to achieve a BLA filing and advancement towards the goal of obtaining marketing approval
- Logical bridge to AAWG; demonstrating CIRM's commitment to Access & Affordability



#### Who is 'new' CLIN4 for?

#### **Eligibility** Criteria:

- Must have an active CLIN2 award
- Must have completed 50% of milestones of an active CLIN2 award
- Must have completed an End-Of-Phase 2 meeting or equivalent with FDA and have concurrence on requirements for a BLA filing

#### What will 'new' CLIN4 provide?

Up to \$12M to cover Late-Stage Development activities necessary for BLA filing but not included in the CLIN2 award



## 'New' CLIN4 Concept Plan

#### Late-Stage Development Activities that may be Funded under CLIN4:

- Activities necessary for filing a BLA such as:
  - conduct of a Pre-BLA meeting with FDA
  - compilation of an electronic common technical document (eCTD)
- Product manufacturing activities necessary to submit a BLA
- Commercial development activities such as pharmacoeconomic analysis, budget impact models (managed health-payer perspective)
- Development of a supply chain strategy
- Initiation of pre-commercialization activities such as production of a Payors' costeffectiveness Analysis Report, compilation of an AMCP Dossier
- Compassionate use of the investigational therapy for patients in the period after enrollment closed and Market Approval is awarded with FDA approval and agreement with the drug product supplier



### Clinical Programs Awards Overview



Aim / Scope

**Eligibility** 

**Duration** 

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CLIN<sub>1</sub>

**IND** Enabling

Pre-IND Meeting
/ Start in 45 Days

24 Months\*

6M to 7.5M\*\*
4M to 5M

CLIN 2

Clinical Trial

Active IND / Start in 45 Days

48 Months

15M

CLIN 4

**BLA Enabling** 

Active CLIN2 /End of Phase 2 Mtg

48 Months

**12M** 

Up to 6 months additional for start-up activities

\*\* \$7.5M for Non- profit; \$5M for Profit





# CIRM requests ICOC approval of these amendments and the CLIN4 concept plan