

# CIRM

CALIFORNIA INSTITUTE FOR  
REGENERATIVE MEDICINE

# Strategic Assessment of CIRM's Clinical Portfolio

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# Outline

## *Open Session*

- **Context**
- **Problem Statement**

## *Closed Session*

- **State of Clinical Portfolio – See Appendices**

## *Open Session*

- **Staff Recommendation**
- **Request for Motion**
- **Board Discussion**

# Presentation Overview

- 1 **Context**
- 2 Problem Statement
- 3 State of Clinical Portfolio
- 4 Staff Recommendation
- 5 Board Discussion

# Our Mission

To accelerate world-class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and the world

# CIRM's Impact Goals

## Accelerating Discovery & Translation

1. **Catalyze** the identification and validation of at least 4 novel targets and biomarkers, ensuring integration into preclinical or clinical research for diseases in California
2. **Accelerate** development and utilization of 5-8 technologies that have the potential to improve safety, efficacy, and/or quality of cell and gene therapies

## Cell & Gene Therapy Approvals

3. **Advance** 4-7 rare disease projects to BLA
4. **Propel** 15-20 therapies targeting diseases affecting Californians to late-stage trials

## Accessibility & Affordability of CIRM-Funded Cell & Gene Therapies

5. **Ensure** that every BLA-ready program has a strategy for access and affordability

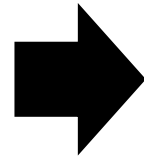
## Diverse Workforce Development

6. **Bolster** CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine

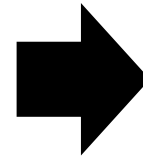
# CIRM's Clinical Funding is Designed to Move Therapies to Patient Impact

**CIRM Mission**  
**Prop 14 A&A Mandate**  
**SAF Goals 3 & 4**

**CIRM Pipeline Programs**  
CLIN2, PDEV, RAPID



**BLAs**



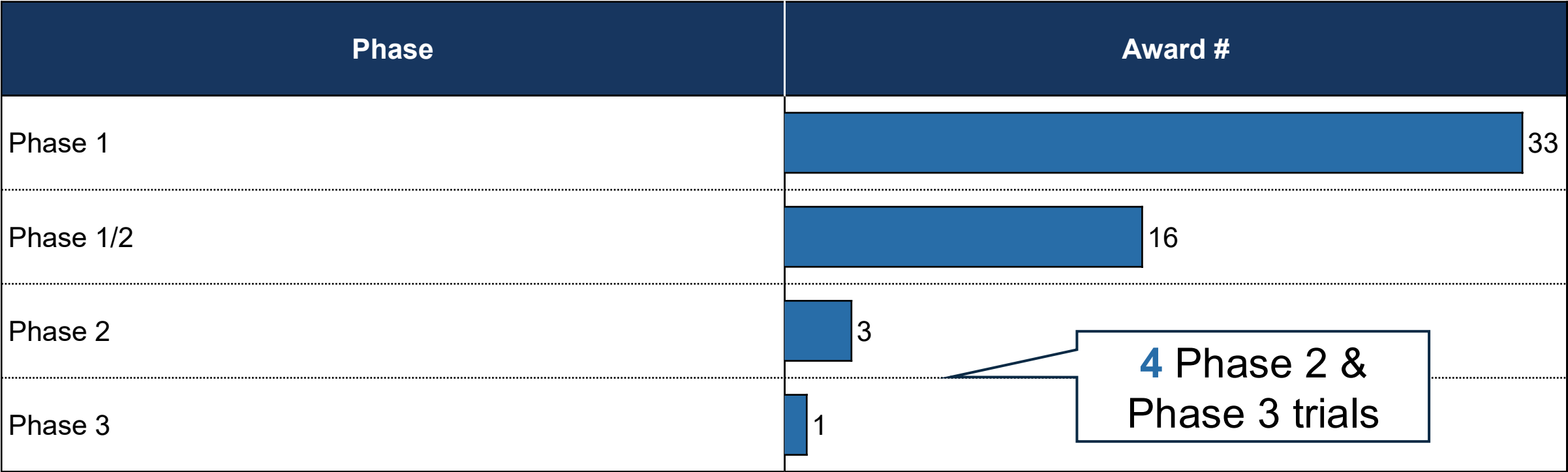
- **Launch & Commercialization**
- **Access for Californians**

## Goal 3: Summary of Progress

CIRM-supported programs are progressing toward BLA

- ① Approved BLA (Kresladi)
- ④ Submitted BLA or planning BLA submission (enrollment complete)
- ⑦ In pivotal trials and/or progressing externally

# Goal 4: Prop 14 Clinical Trials



## CIRM's Historical Clinical Portfolio Shows External Validation



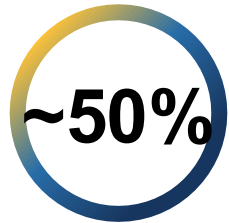
121

**CIRM-supported clinical trials**



112

**Unique CIRM-supported clinical programs**



~50%

**Programs secured external capital**



~11

**Active programs with current potential commercialization capacity**

External capital refers to venture or public-market financing; Commercialization-capable count reflects therapies backed by companies with funding to commercialize the candidate if clinically successful

**... But external capital does not ensure BLA path**

### **Caveats:**

- External capital  $\neq$  BLA path
- Company financing  $\neq$  capital committed to the CIRM-supported asset
- Commercialization capacity  $\neq$  guaranteed launch or access

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# What CIRM's Clinical Portfolio Is Revealing

## What happens after initial CIRM-funded clinical development

### Some programs continue

- Generate enough evidence for others to carry development forward
- CIRM stays engaged on access, CA impact, and learnings

### Some programs stall

- Reach meaningful clinical development but lack viable path forward
- CIRM would need to assume substantially more of the remaining development burden

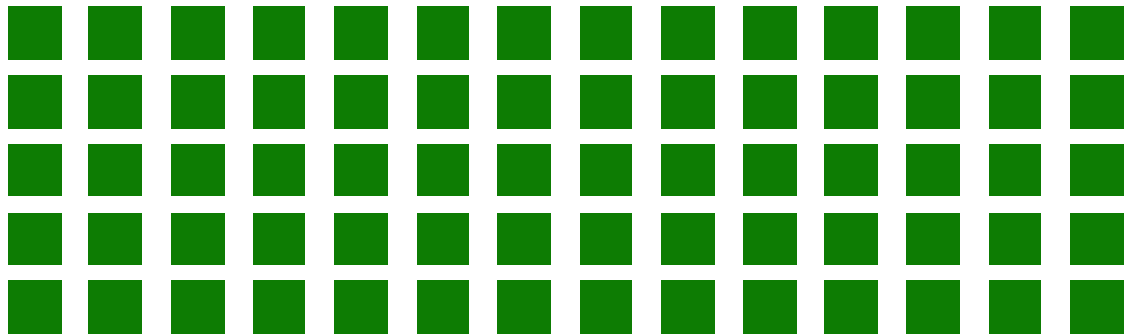
**What role** should CIRM play in clinical development?  
**Where should CIRM invest** to maximize therapies that ultimately reach patients?

# What Should CIRM's Remaining \$1.054B Accomplish?

~\$1.054B remaining clinical-development horizon

## Broader portfolio / external leverage

~\$10–15M per program  
~70–105 awards



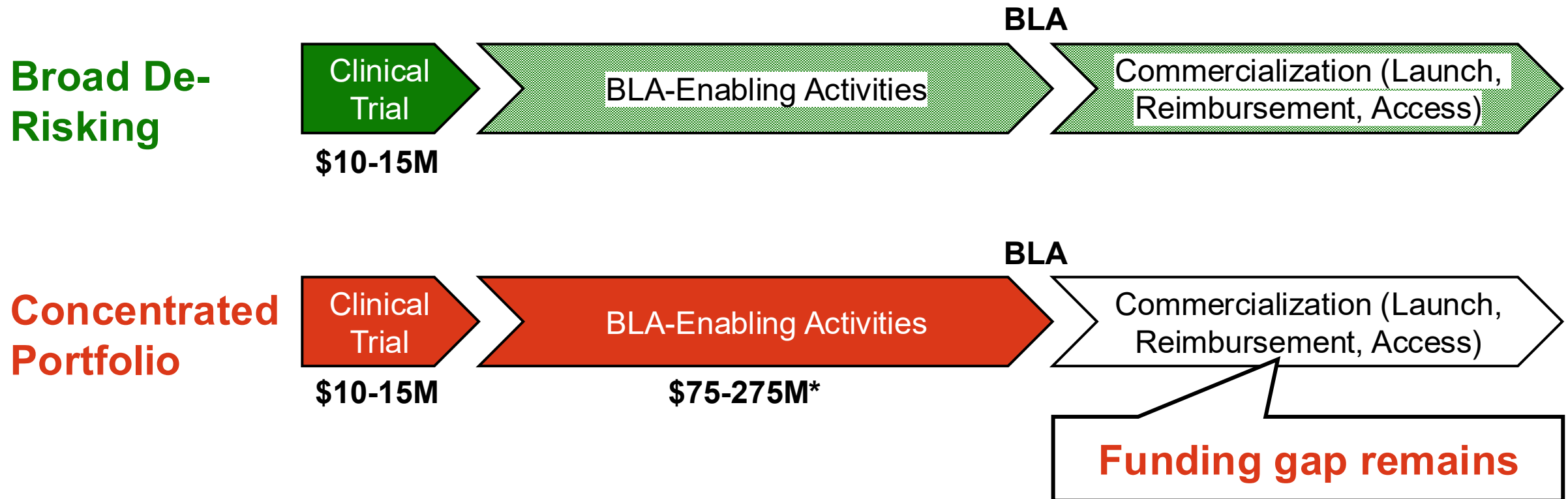
## Concentrated portfolio / CIRM carries development

~\$90–300M per program (up to BLA)  
~4–11 awards



**Concentrated late-stage** funding comes at the **cost of more shots on goal**

# Commercialization Gap Despite Focused Funding for BLA



\*Approximate; actual depends on modality, indication, patient population, etc.

# Problem Statement

## The Portfolio Analysis Raises A Role Question For CIRM

- Many late-stage programs are advancing toward pivotal development and BLA *without additional* CIRM funding
- Other programs have reached a point where continued development requires *substantial* CIRM funding

### Questions for the Board:

- What **role** should CIRM play in funding clinical programs?
- Should CIRM continue as the primary funder through **late-stage development and BLA**?

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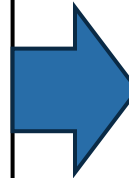
# Scope of Today's Analysis

Focus is on programs where CIRM's future role is most relevant

Historical Clinical Portfolio

**112**

Unique clinical programs



Closed Session Analysis

**53 + 6**

**Active** clinical-stage awards + closed programs  
with confirmed **ongoing** development

# Three Types of Clinical Programs

## Programs Categorized by General Path Forward

Path to BLA w/o Additional  
CIRM Support

Unlikely Path to BLA w/o CIRM  
Support

Need for CIRM Support TBD

# Criteria for Program Types

## Path to BLA w/o Additional CIRM Support

- Confirmed ongoing development towards BLA
- Sufficient external funding (Big Pharma or VC) to carry program to BLA & beyond

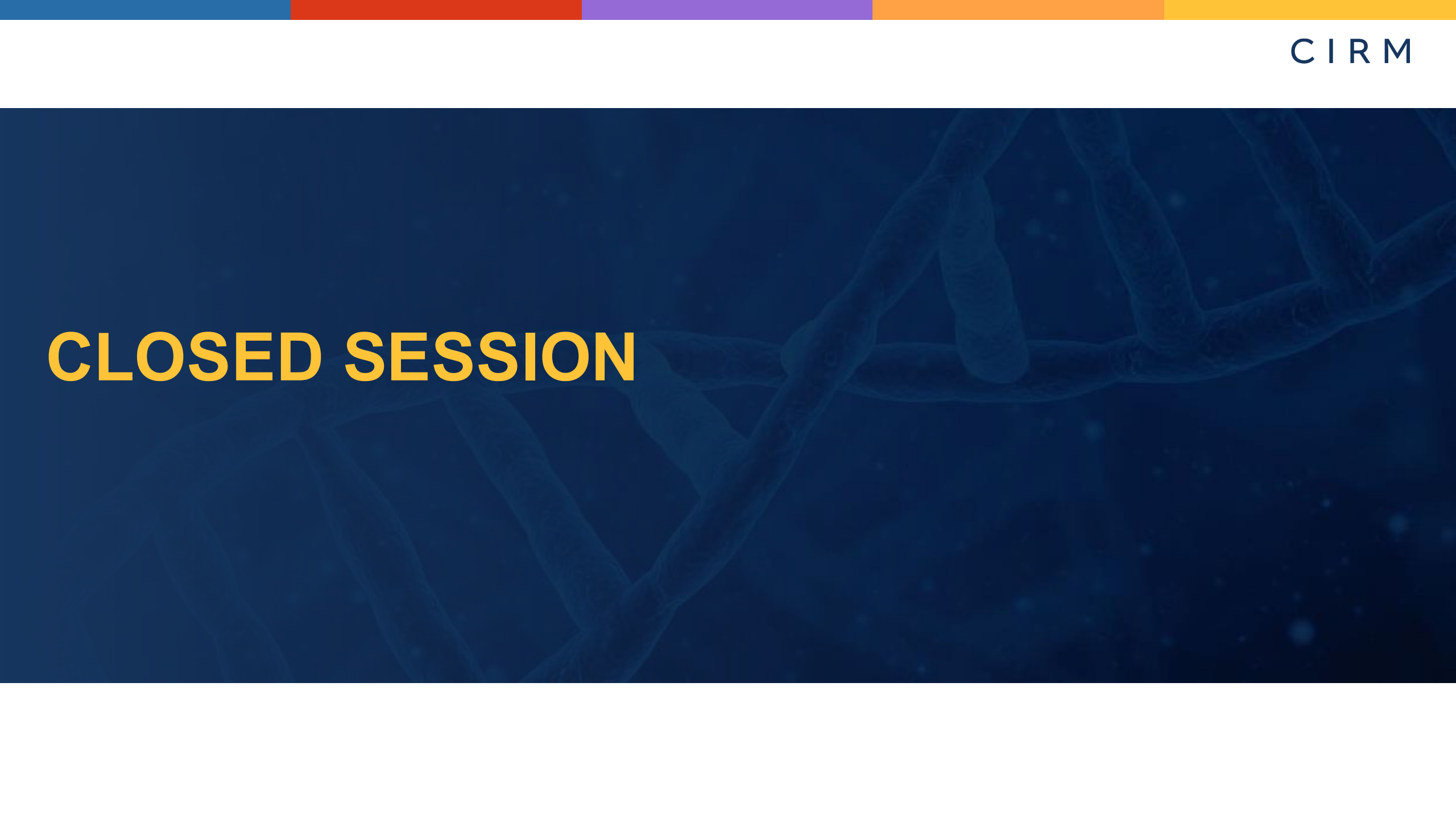
## Unlikely Path to BLA w/o CIRM Support

- Currently in pivotal trials
- Insufficient/no external funding to carry program forward

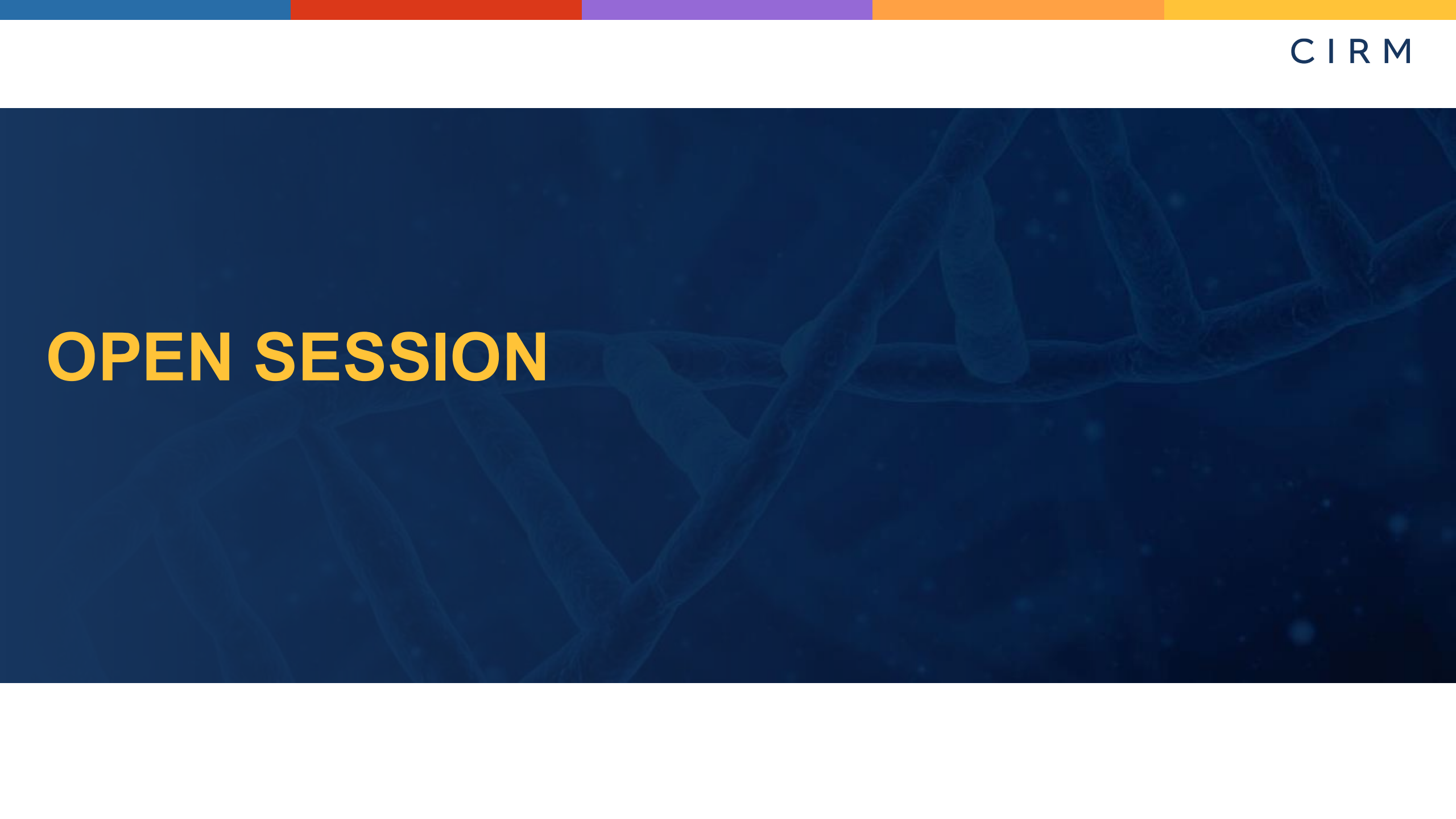
## Need for CIRM Support TBD

- Mixed group with 3 types:
  - Too early to know – waiting on clinical data
  - Promising but financing-dependent
  - Already showing a difficult path ahead

# CLOSED SESSION



# OPEN SESSION



# Presentation Overview

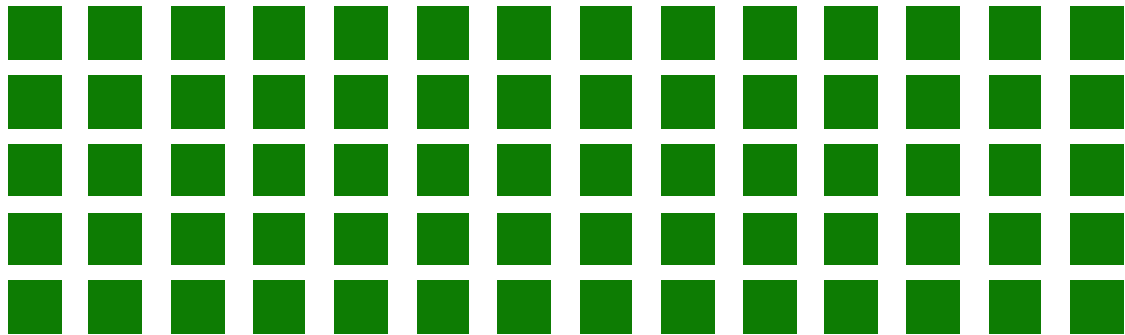
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# Recap: What Should CIRM's Remaining \$1.054B Accomplish?

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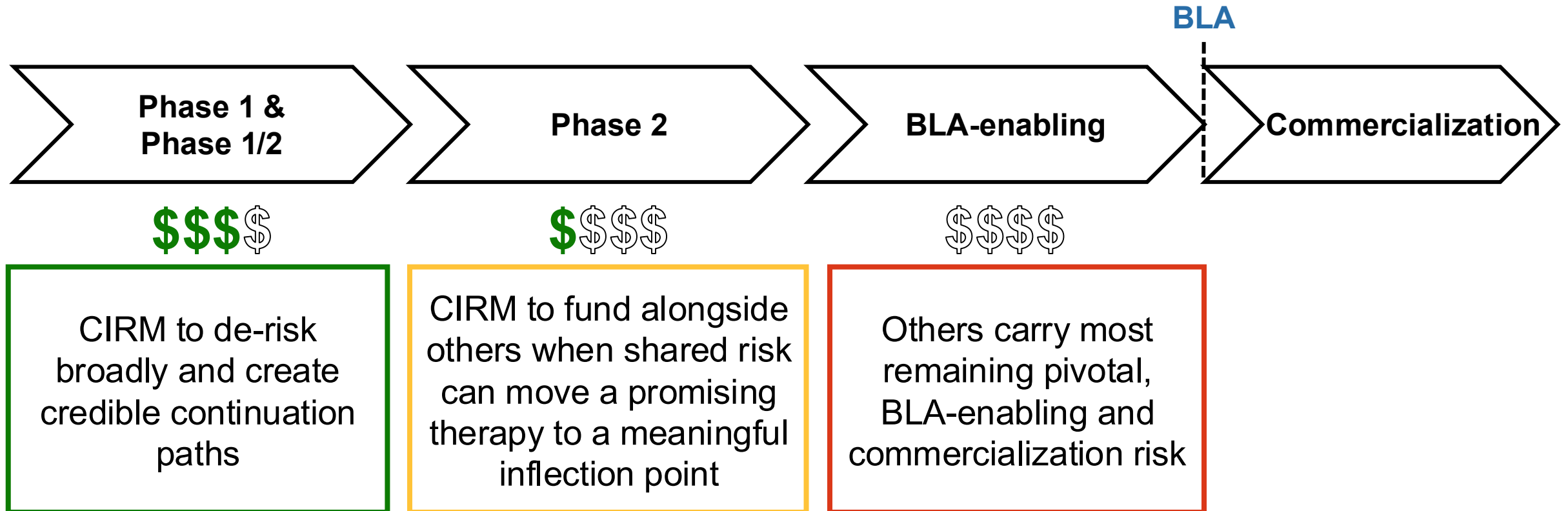
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# Staff Recommendation

## Focus Clinical Funding on De-Risking & Credible Continuation



## Board Direction Requested

If the Board supports this direction, CIRM Staff will return in December with:

### Clinical Program Updates

- Updated CLIN2 concept with:
  - Recommended refinements to scope and funding approach
  - Clearer expected outcomes for CIRM-supported clinical programs
  - Updates to program requirements
- Proposal for a more structured approach for portfolio visibility and external engagement

### Review Process Updates

- Review criteria aligned with the proposed clinical funding approach
- Plans for recruitment of new GWG reviewers aligned with updated CLIN2 focus
- Plans for training GWG reviewers



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# Requested Action

CIRM requests the ICOC approve these recommendations

# Board Discussion