Clinical Development Awards (CLIN2)

Informational Webinar

May 30th, 2025

CIRM
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Quick Announcements

- Submissions **are due June 30, 2025.**
- Webinar recording and FAQ document will be posted in ~1-2 weeks
- Q&A will focus on pre-submitted questions - specific programmatic questions may be added to the Q&A chat or directed to clinical@cirm.ca.gov and will be addressed in the FAQ mentioned above

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Agenda

1 Part 1: Presentation — 2:30 - 3:10pm

- Program Overview
- Application Process
- Information and Resources for Applicants

2 Part 2: Question & Answer — 3:10 - 3:30pm

Program Overview

Our Mission

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

CIRM's Strategic Allocation Framework

The **Strategic Allocation Framework (SAF)** - Structured and data-driven approach to prioritize resource allocation and provide recommendations to the ICOC for continued implementation of CIRM's strategic plan

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

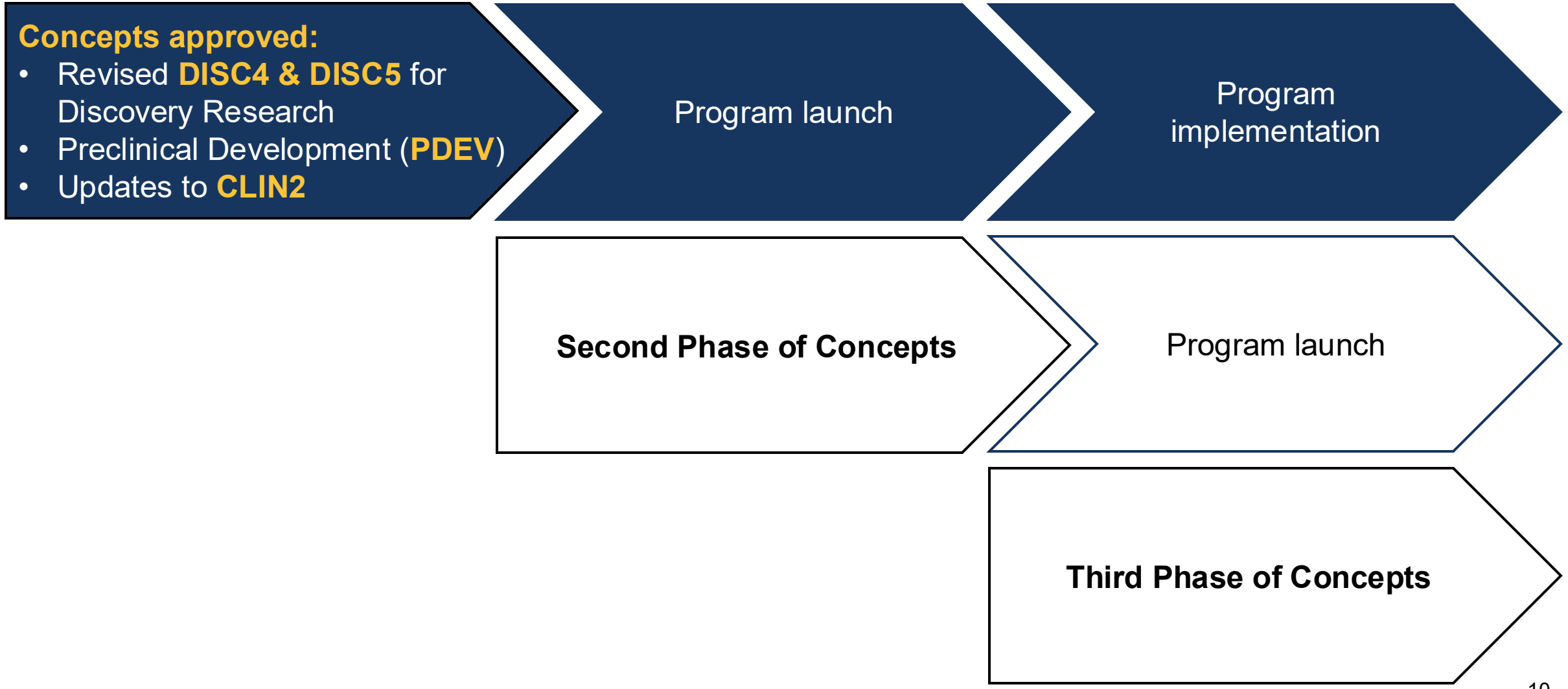
Accessibility & Affordability of CIRM-Funded Cell & Gene Therapies

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

SAF Implementation: 3 Phases



CLIN2 | Prioritizing to achieve SAF Goal

SAF Goal 4: Propel 15-20 therapies targeting diseases affecting Californians to late-stage trials

To achieve the SAF goal, the CLIN2 Program will incorporate program preferences

Guiding Principles:

- Fund therapies that
  - Offer potential for transformative clinical impact
  - Have a well-developed plan for access and affordability

Implementation Plan:

- Build a diverse portfolio of therapeutic approaches
- Priorities informed by internal portfolio and external landscape analyses
- Approved on a fiscal year basis by the ICOC

Scope and Expected Outcomes

Supports completion of an interventional phase 1, 2 or 3 clinical trial for a stem-cell based or genetic therapeutic candidate and may also support an associated natural-history comparator or lead-in normal healthy volunteer study

Projects should aim to achieve the following outcomes:

- Progress a therapeutic candidate toward a later stage trial and eventual BLA or NDA filing
- Create a well-developed plan for ensuring access and affordability

Projects should have the potential to address significant unmet medical needs and provide transformative benefits to patients, families and the healthcare system.

CLIN2 | Award Structure

	CLIN2		
	First-in-Human	**Phase 2 or subsequent***	**Phase 3 or pivotal**
Recurrence	4x per year		
Max Duration	4 years		
Applicant	California or non-California organizations		
Co-funding**	30% (for-profit**) None (non-profit)	50% (for-profit**) None (non-profit)	50%
Max Award (Total Cost)	$8M (for-profit) $12M (non-profit)	$15M	$15M

* “Subsequent” trials are any phase 1 trials following a First-In-Human trial in the proposed disease indication and using a given route of administration.
**Co-funding requirement also applies to for-profit partners of non-profit applicants.

CLIN2 | Eligibility

	Eligibility Requirements
Applicant	• California and non-California organizations
Eligible Candidates	• Stem cell-based therapies and genetic therapies
Candidate Readiness	• New program to CIRM: IND cleared by FDA before CLIN2 application • CIRM pipeline program*: IND filed before CLIN2 application and cleared by FDA within 30 days of CLIN2 application submission
Expected Outcome	• Completion of a clinical trial and program prepared to advance to next stage
Award Start	• Must be ready to start within 60 days of award approval
PI/PM Effort	• PI – 15% average maintained through duration of award • PM – 50% average maintained through duration of award
Co-Funding**	• FIH: 30% (For-profit***) • Ph2 or Ph1 subsequent to FIH: 50% (For-profit***) • Ph3 or pivotal: 50% For-profit or Non-profit

* Pipeline program: progressing from an IND-enabling stage or earlier phase clinical trial CIRM award
** Co-funding is a percentage of total allowable project costs
***Or for-profit partners of non-profits

CLIN2 | Preferences for FY25/26

Preferences will be factored in during Qualification and ARS review

Concept Preferences	Rationale
Pluripotent stem cell-derived therapies	• Propositions 71 and 14 • Potential to address patient access & affordability barriers
In vivo genetic therapies	• Potential to address patient access & affordability barriers
Non-viral nucleic acid delivery	• Potential to address patient access & affordability barriers
Diseases of the brain and CNS (Prop 14)	• Proposition 14 priority
CA organizations	• CA taxpayer-funded initiative
Progressions from IND-enabling or pipeline trial awards	• Advance CIRM-funded therapies
Fast Track, RMAT, or Breakthrough designations	• Leverage greater FDA access
Pivotal trials	• Fastest route to BLA

CLIN2 | Required Activities

- ✓ Clinical operations activities to complete the trial on time
- ✓ Outreach, enrollment, and retention activities to achieve trial enrollment demographics reflecting the target patient population
- ✓ Treatment of patients with the therapeutic candidate (or control) and follow-up visits per the clinical protocol
- ✓ Sharing of non-clinical and clinical data per the CIRM data sharing requirements
- ✓ Activities associated with access and affordability planning for the therapeutic candidate in the proposed indication

***Not an exhaustive list; see PA for further details**

CLIN2 | Allowable activities

- ✓ Natural history studies needed for baseline or control data for the interventional trial
- ✓ Lead-in studies in normal healthy volunteers for the interventional trial
- ✓ Studies to develop biomarkers, understand mechanisms of action and develop a potency assay
- ✓ Manufacturing activities to supply the current clinical trial, including technology transfer and FDA-approved comparability studies, if needed
- ✓ Manufacturing for the next phase trial. Conditionally allowable based on...
  - ✓ An interim evaluation by CIRM and independent experts of the clinical trial data
  - ✓ Provision of 50% co-funding for this activity, if co-funding is required as specified in "Award Structure" slide

***Not an exhaustive list; see PA for further details**

CLIN2 | Out of Scope Activities (CLIN2 will NOT fund)

- ✗ Costs incurred on or before the date of ICOC approval
- ✗ Discovery or translational research
- ✗ Activities already budgeted or paid for under a prior, existing or future CIRM award
- ✗ The costs of activities performed by a separate out-of-state organization that retains intellectual property or independent publication rights in any intellectual property (e.g., invention, technology, data) arising out of the CIRM-funded project

***Not an exhaustive list**

Access and Affordability Strategic Planning

Access and Affordability Strategic Planning	
Purpose	• Ensure that every BLA-ready program has a strategy for access and affordability by incorporating stage-appropriate activity planning
Resources	• Activity checklist and descriptions
At Application	• Describe progress and outcomes for stage-appropriate prerequisite activities • Describe plans for stage-appropriate award activities
Award Management	• Access and affordability activities incorporated into milestones • Requirement to report on additional activities, including final strategic report

Scientific Review Criteria

1. **Value Proposition**: Assess potential to meaningfully improve clinical outcomes for the intended population, and to have uptake of the therapy by patients, payors and providers.
2. **Rationale**: Assess the robustness of the scientific rationale and supporting evidence, including justification for the indication, therapeutic approach, regimen and route of administration.
3. **Project Plan and Design**: Evaluate whether the proposed activities are appropriate to efficiently and effectively drive clinical development of the proposed therapy.
4. **Project Team and Resources**: Evaluate whether the team's leadership, expertise, staffing and resources are sufficient to ensure successful completion of the project.
5. **Population Impact**: Evaluate the extent to which the project considers the impact of the therapy across the affected population and is designed to achieve representative trial participation.

CIRM Data Sharing Requirement

https://www.cirm.ca.gov/how-to-apply/

1. **Develop a Data Sharing and Management Plan (DSMP)**
   - Metadata Catalog
   - Data Use Limitations (DUL) Institutional Certification
   - Questionnaire

2. **Deposit data in data repositories by end of award**

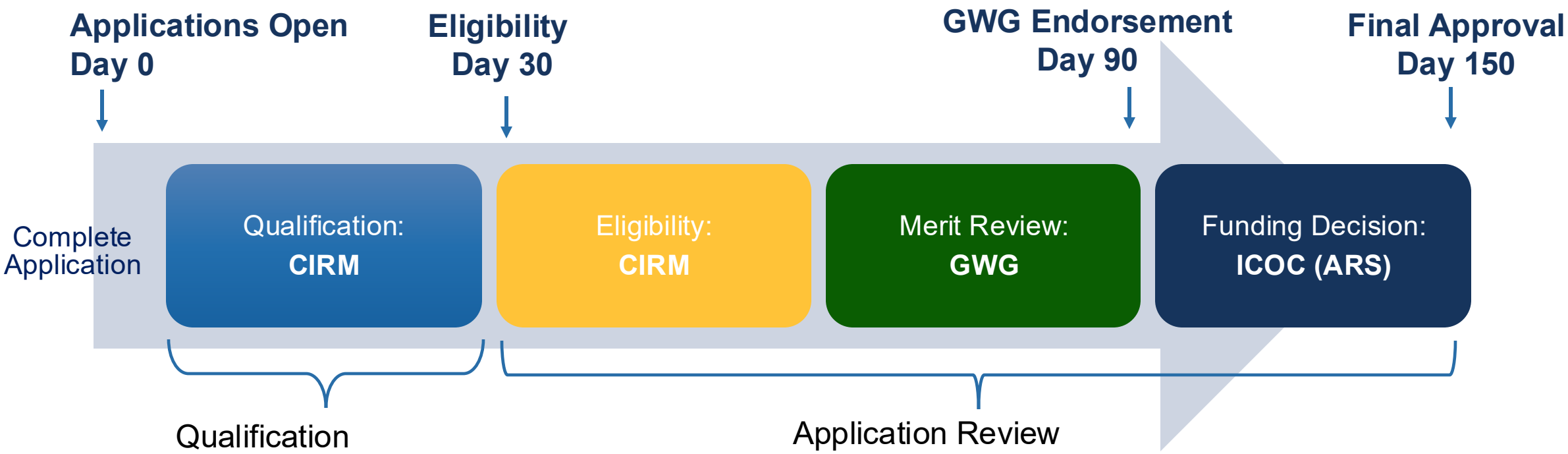
3. **Trial results must be submitted to ClinicalTrials.gov 12 months from the Primary Completion Date**

Application Process

CLIN2 Landing Page

Visit cirm.ca.gov/clin2/ to learn more about our CLIN2 funding opportunity

Overview of CLIN2 Qualification and Review Process



Is the proposal aligned with CLIN2 preferences?

Can the application be reviewed?

Is the application scientifically meritorious?

Has access and affordability been considered?

Should CIRM fund this project?

Application | Getting Started

- Log-in at http://grants.cirm.ca.gov/
- Click on “Open Programs”

Other Things You Could Do

- Browse current Open Programs to start a new application
- Review Your Applications to see all previously submitted, expired, abandoned, and withdrawn applications

- Select “Start a submission form for prospective CLIN2 applicants”

Open Programs

RFAs and Programs Open For Applications	Actions
CLIN 2 CIRM Clinical Trial Stage Projects: CLIN2 Grant Application	Start a CLIN2 Grant Application
DISC4 PSUB: Pre-submission form for prospective DISC4 applicants	Start a Pre-submission form for prospective DISC4 applicants
The PI/PD of all applications/pre-applications/LOIs created here will be the person whose name is listed at the top of this page. You cannot use these links to create applications for any other PI/PD.	

Application | Components

ONLINE SECTION

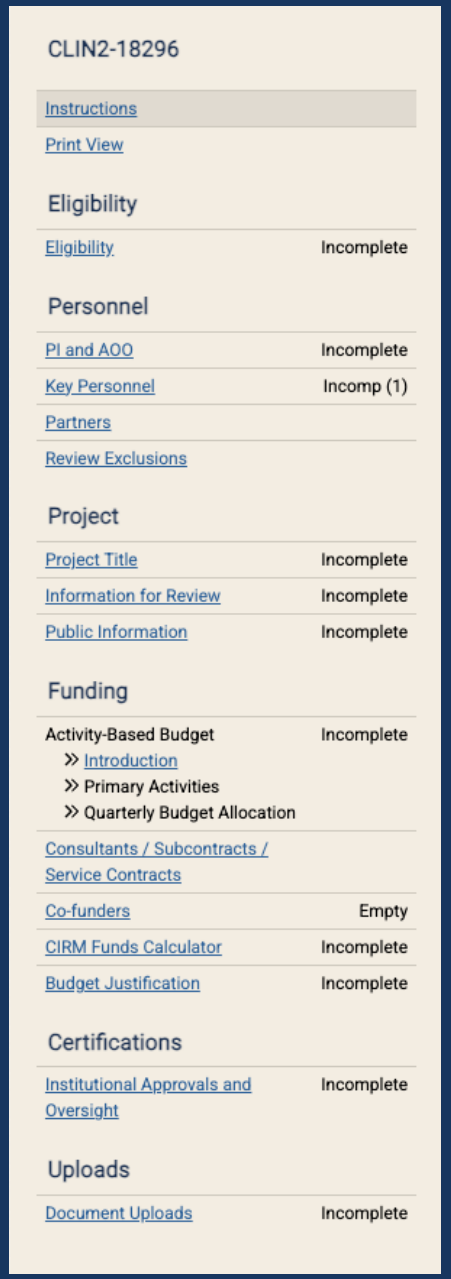
- Eligibility
- Personnel (Designating PI & AOO)
- Funding (ABB)

UPLOADS SECTION INCLUDES

- Proposal – template provided
- FDA Correspondence
- Access and Affordability checklist
- Additional uploads dependent on applicant criteria

All sections are required.

Annotation will change from "Incomplete" to "Complete".



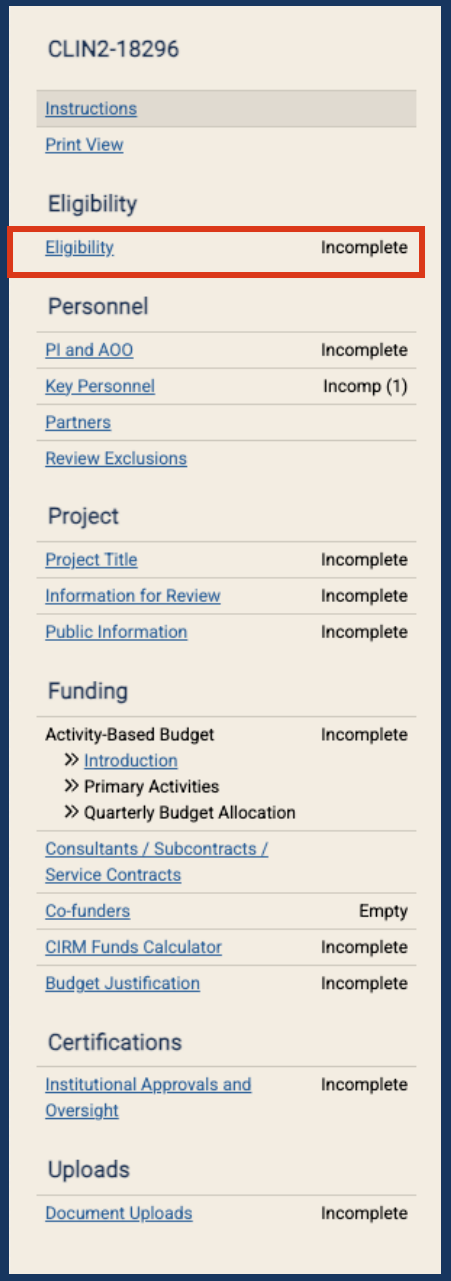
Application | Eligibility

Requirements

- Must have regulatory approval to proceed with proposed trial
- The project team must include an experienced Project Manager and Data Project Manager
- Must have at least one trial site located in California

Certifications

- Documentation of proof of Co-funding
- Declare applicant as a CA or Non-CA organization
- Verify that the PI is eligible
- Certify that the applicant is the IND holder



Application | PI and AOO

Principal Investigator

- Must commit at least 15% effort and adhere to CIRM's requirements

Authorized Organizational Official ("AOO")

- The AOO is the individual who is authorized to act for the applicant organization and to assume the obligations imposed by the laws, regulations, requirements, and conditions that apply to Applications and Awards
- Each awardee organization must have at least one active AOO
- All CIRM applications require an AOO be confirmed prior to submission

Application | ABB

Activities-Based Budget ("ABB")

- CLIN2 applicants must complete activities-based budgets as part of their application

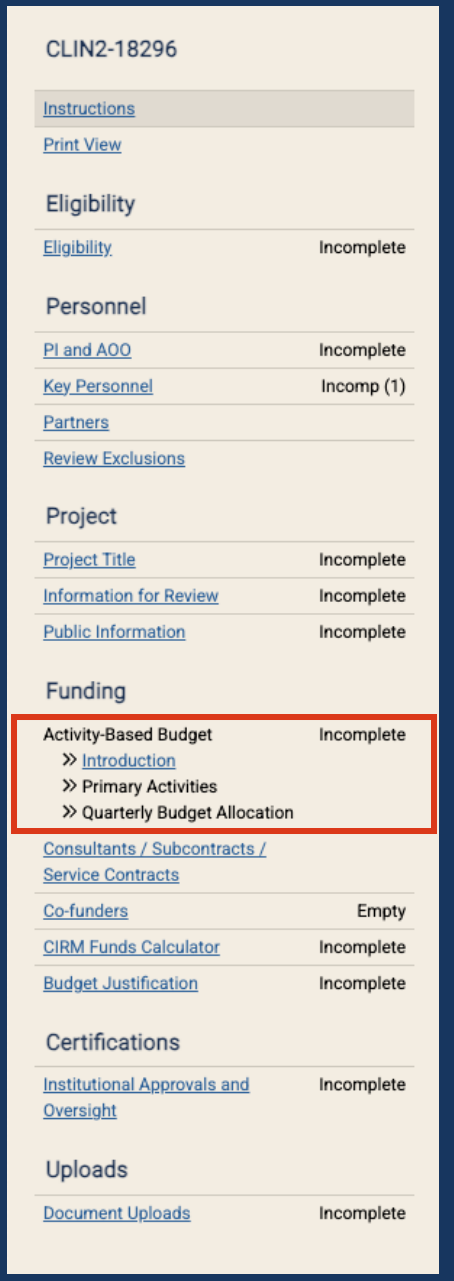
Introduction

- Expense reporting structure (single site vs multi site)
- Declare whether project includes unallowable costs

Primary Activities

- CMC, Clinical and Non-clinical activities

Quarterly Budget Allocation



Co-funding Requirements | Calculation

- CIRM will require co-funding from the applicant based on the total “Allowable Project Costs”
- Allowable Project Costs are those costs permitted under CIRM policies and regulations and include direct, facilities and indirect costs
- The sum of CIRM funds requested plus the co-funding contribution by the applicant make up the total Allowable Project Costs
- For non-CA applicants, co-funding includes both the required co-funding on Allowable Project Costs and the unallowable non-CA costs portion
- Note that the GMS ABB automatically calculates required co-funding

Contacts and Deadlines

Submissions due by June 30, 2025 at 2 p.m. PDT

Programmatic Questions
clinical@cirm.ca.gov

Review Questions
review@cirm.ca.gov

Budget and Grants Admin Questions
grantsmanagement@cirm.ca.gov

Clinical

www.cirm.ca.gov/CLIN2/

Please reference documents on our website for guidance

Webinar and FAQ will be posted within 1-2 weeks

Information and Resources for Applicants

Alpha Clinics

CIRM ALPHA CLINICS NETWORK

CIRM's Alpha Clinics are composed of **9 leading California medical centers**:

- World-class medical facilities specializing in cell & gene therapies
- Promoting patient access through patient engagement & outreach
- Transformative research

Cedars Sinai
City of Hope
UCDAVIS HEALTH Alpha Stem Cell Clinic
Stanford MEDICINE
Alpha Clinic UCSF University of California San Francisco
UCLA Health Alpha Clinic
UCI Alpha Clinic
USC+CHLA Alpha Clinic
UC San Diego SANFORD STEM CELL INSTITUTE Sanford Stem Cell Clinical Center

Clinical Research Dashboard*:

- **337** Clinical Trials
- **2000+** Patients Treated
- **40+** Diseases

CIRM Manufacturing Network

CIRM's INFR5 Program funds **9 academic Good Manufacturing Practice (GMP) facilities** in California to help **overcome manufacturing bottlenecks**

Goals of the network:

- Accelerate and de-risk path to commercialization
- Advance standards and quality by design
- Build manufacturing leadership and workforce

CIRM-funded awards supported*:
- **30** Preclinical-stage
- **19** Clinical-stage

Network Members

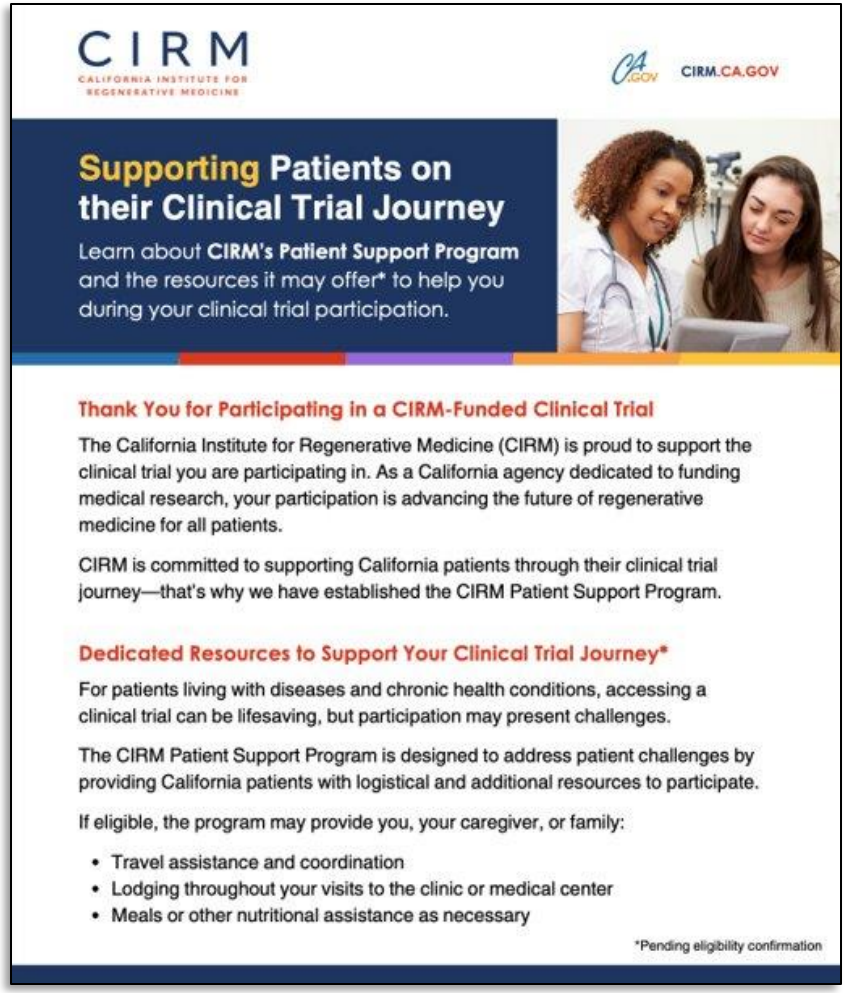
Cedars Sinai
City of Hope
Stanford University
UC DAVIS University of California
UCI University of California, Irvine
UCLA
UC San Diego
UCSF
USC University of Southern California

Patient Support Program

The Patient Support Program (PSP) provides **logistical and financial support to California patients** enrolled in **CIRM-funded clinical trials**

The program offers:

- Travel assistance and coordination
- Lodging support throughout clinical visits
- Meals and other nutritional assistance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Supporting Patients on their Clinical Trial Journey

Learn about **CIRM's Patient Support Program** and the resources it may offer* to help you during your clinical trial participation.

Thank You for Participating in a CIRM-Funded Clinical Trial

The California Institute for Regenerative Medicine (CIRM) is proud to support the clinical trial you are participating in. As a California agency dedicated to funding medical research, your participation is advancing the future of regenerative medicine for all patients.

CIRM is committed to supporting California patients through their clinical trial journey—that's why we have established the CIRM Patient Support Program.

Dedicated Resources to Support Your Clinical Trial Journey*

For patients living with diseases and chronic health conditions, accessing a clinical trial can be lifesaving, but participation may present challenges.

The CIRM Patient Support Program is designed to address patient challenges by providing California patients with logistical and additional resources to participate.

If eligible, the program may provide you, your caregiver, or family:

- Travel assistance and coordination
- Lodging throughout your visits to the clinic or medical center
- Meals or other nutritional assistance as necessary

*Pending eligibility confirmation

Q&A