Preclinical Development Awards (PDEV)

Informational Webinar

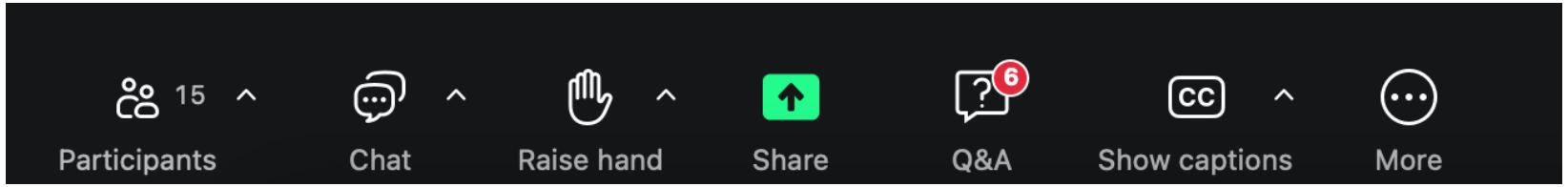
May 22, 2025, 11:00 AM PST

CIRM
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

Quick Announcements

- This webinar pertains to Preclinical Development (PDEV) overview & pre-submission process. Pre-submissions are due **June 12, 2025**

- Q&A will focus on general questions. If you have questions related to your specific project, please submit them to preclinical@cirm.ca.gov
  - Use the Q&A box on Zoom to enter questions
  - Please save your questions until AFTER the presentation, we will not answer any questions during the presentation



Disclaimer: This webinar highlights key information only

Please refer to contents on the program page for comprehensive information

- Program Announcement
- Applicant-facing resources
- Final FAQ

https://www.cirm.ca.gov/preclinical/

Preclinical Development Team

Shyam Patel
Associate Vice President
Preclinical Development

Ross Okamura
CIRM Fellow
Preclinical Development

Jim Campanelli
Sr. Science Officer
Preclinical Development

Dongjin Lee
Sr. Science Officer
Preclinical Development

Lisa McGinley
Sr. Science Officer
Preclinical Development

Thomas Trinh
Project Manager
Preclinical Development

Webinar Panelists

Shyam Patel
Associate Vice President
Preclinical Development

Rosa Canet-Aviles
Chief Science Officer

Jim Campanelli
Sr. Science Officer
Preclinical Development

Lisa McGinley
Sr. Science Officer
Preclinical Development

Thomas Trinh
Project Manager
Preclinical Development

Janie Byrum
Sr. Science Officer
Data Infrastructure

Hayley Lam
Director
Portfolio Development & Review

Doug Kearney
Director
Grants Management

Agenda

1 Part 1: Presentation — 11:00 - 11:30am

- Context
- Program Overview
- Data & Knowledge Sharing Requirements
- Review Process & Criteria
- Pre-submission Components & Considerations

2 Part 2: Question & Answer — 11:30 - 12:00pm

Context

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

ICOC – Independent Citizen Oversight Committee; CIRM's governing board

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

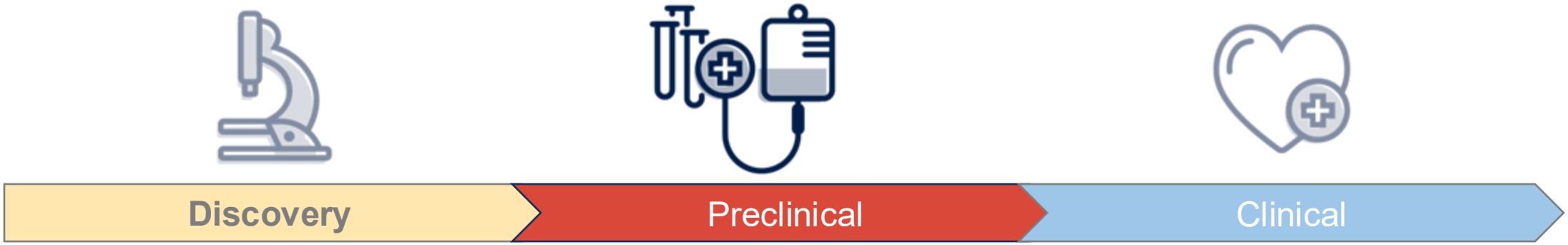
6. **Bolster** CIRM's workforce development programs to address gaps and meet evolving demands in regenerative medicine

Recommendations to Achieve Goal 4

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

Streamline Preclinical Development Programs

- Consolidate DISC2, TRAN1-4, and CLIN1 to accelerate the preclinical development incentivizing multidisciplinary collaborations and rapid progression to IND
- Incorporate prioritization of innovative therapies for diseases that affect Californians



SAF Implementation: 3 Phases

Concepts approved:
- Revised **DISC4 & DISC5** for Discovery Research
- Preclinical Development (**PDEV**)
- Updates to **CLIN2**

Program launch

Program implementation

Second Phase of Concepts

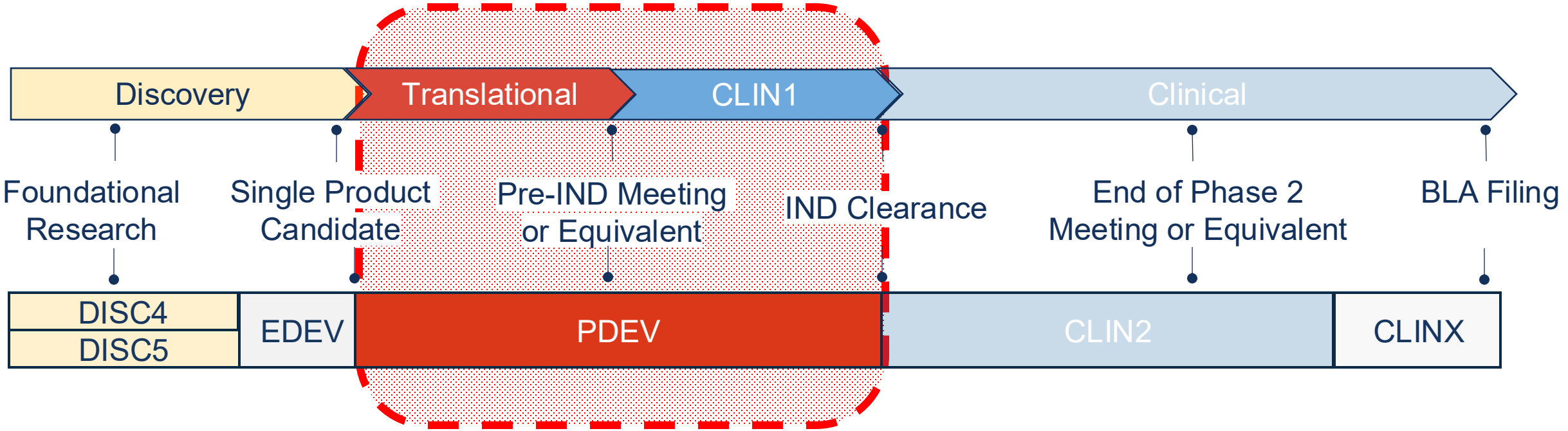
Program launch

Third Phase of Concepts

Program Overview

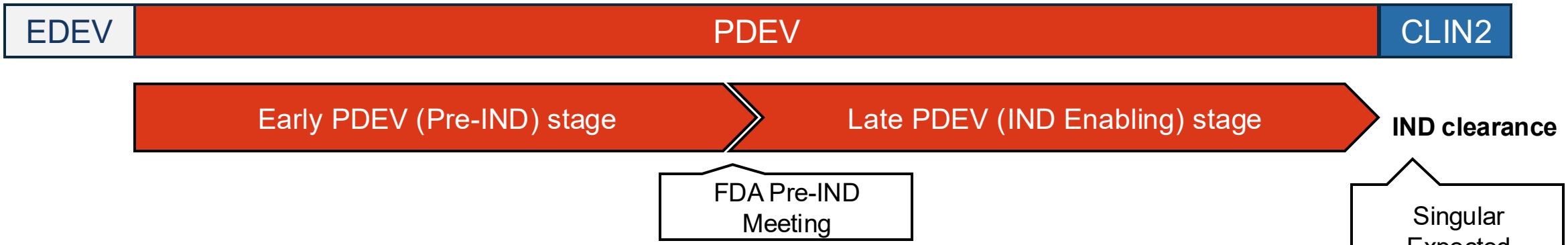
Overview

PDEV combines TRAN1 and CLIN1 into one program with a singular **objective** of **accelerating stem cell-based and genetic therapies to first-in-human clinical trials**



PDEV | Flexible Entry Points with a Single Outcome

PDEV covers critical pre-clinical development activities from candidate optimization to trial startup



Illustrative activities *(non-exhaustive)*

CMC	Candidate optimization, formulation, process development	GMP manufacturing of DS & DP, analytical assay development & qualification
Nonclinical	Pilot dose range finding, pilot safety/ toxicology studies, efficacy studies	GLP safety / toxicology studies
Clinical	Clinical plan development/draft protocol, access planning	Clinical protocol development, trial start-up activities, access planning
Regulatory	INTERACT & Pre-IND package development	IND package development

Award Amount & Duration Varies by Stages

EDEV | PDEV | CLIN2

Early PDEV (Pre-IND) stage → Late PDEV (IND Enabling) stage → **IND clearance**

Max Stage Amount: $5.5M

Max Stage Duration: 30 months[1]

Max Stage Amount: $7.5M

Max Stage Duration: 30 months[2]

Maximum Award Amount: $13M

Maximum Award Duration: 5 years

[1] Inclusive of maximum optional 6 months for candidate optimization
[2] Inclusive of maximum optional 6 months for trial startup activity following IND clearance

Program Structure

	PDEV
Recurrence	2x / year
Max Award Duration	5 years
Applicant	California non-profit or for-profit research institutions
Co-funding[1]	20% (cash based or warrants based)
Max Award (total cost)	$13M (Total Project Cost)

[1]Required for for-profit applicants and nonprofits applicants with for profit partners

Eligibility

	Eligibility Requirements
Applicant	• California organization
Eligible Candidates	• Stem cell-based cell therapies and genetic therapies
Candidate Readiness	• Demonstrated disease modifying activity with candidate (same as TRAN)
Expected Outcome	• Must propose activities to achieve clearance of IND submission
Award Start	• Must be ready to start within 90 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[1]	• 20% Total Allowable Project Costs (Cash-based or Warrants-based co-funding)

[1]Required for for-profit applicants and nonprofits applicants with for profit partners

Allowable Activities (PDEV will Fund)

Allowable activities: Early PDEV (Pre-IND) Stage
✓ All activities necessary to ready a human therapeutic candidate for pivotal IND-enabling preclinical studies including preparation and conduct of a pre-IND meeting with the FDA
✓ All activities necessary to optimize a candidate and to confirm disease-modifying activity for the finalized single human therapeutic candidate
Allowable activities: Late PDEV (IND-Enabling) Stage
✓ All IND-enabling activities necessary for submission and clearance of an IND with the FDA for a clinical trial with the therapeutic candidate
✓ Clinical trial startup activities to facilitate eventual rapid recruitment of patients
Allowable activities: Across All Stages
✓ Activities associated with managing, preserving, and sharing data and knowledge from the project
✓ Activities associated with market access strategic planning, including addressing barriers to patient access and affordability

Out-of-Scope Activities (PDEV will NOT fund)

Unallowable activities: Across All Stages
X The conduct of a clinical trial beyond start-up activities
X Patient recruitment, screening, or enrollment
X Activities already budgeted or paid for under a prior, existing or future CIRM award
X 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
X Costs incurred on or before the date of ICOC approval

Access and Affordability Activities in PDEV Awards

- **The following required activities will be milestoned**
  - Market landscape assessment/market research
  - Reimbursement and market access strategy

- **The following recommended activities will be reported in progress reports**
  - IP strategy
  - Financial planning and fundraising strategy
  - Regulatory pathway strategy
  - Partnerships and collaborations

Data and Knowledge Sharing Requirements

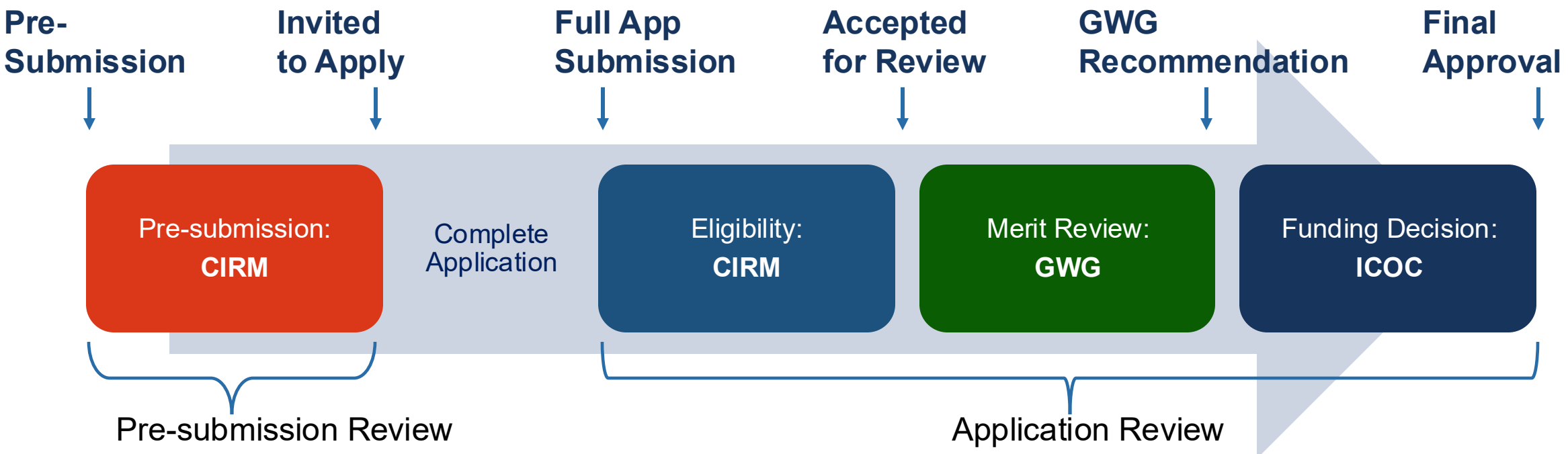
CIRM Data and Knowledge Sharing Requirement

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 applicable data in data repositories by end of award**
3. **Participate in the PDEV knowledge sharing network**

Review Process and Criteria

Overview of PDEV Application Process



Is proposal aligned with CIRM priorities?

Can the application be reviewed?

Is the application scientifically meritorious?

Should CIRM fund this project?

Scientific Review Criteria

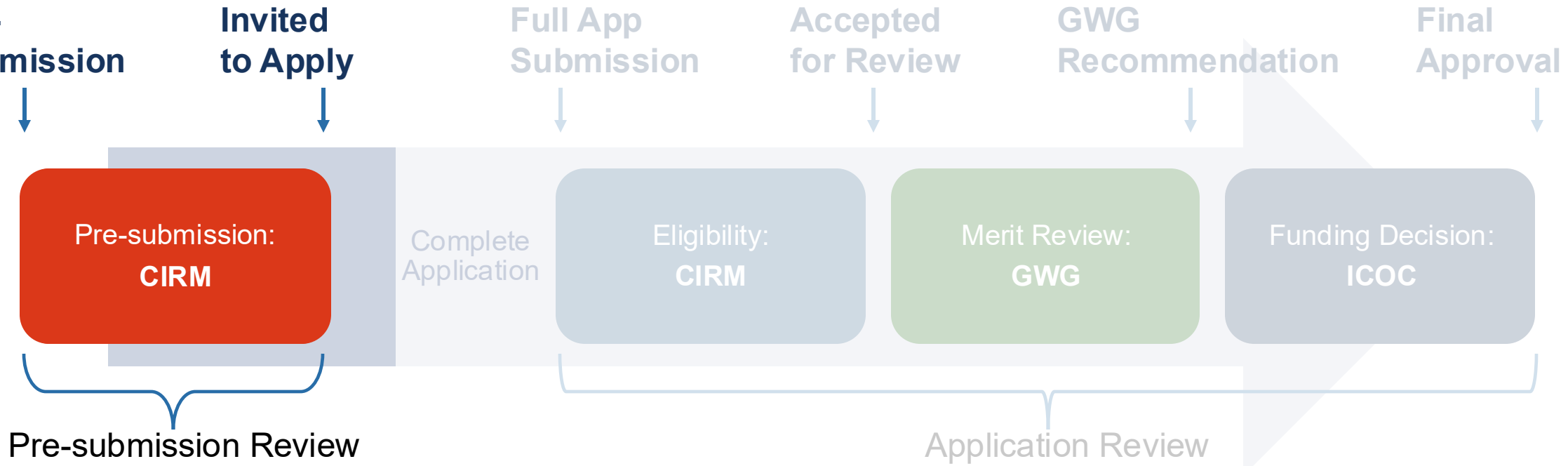
Merit Review

Criteria	Description
Value Proposition	Evaluate the extent to which the therapy offers a compelling value proposition
Rationale	Evaluate the scientific rationale for the proposed therapy and the strength of the supporting data
Project Plan & Design	Evaluate the project's plan and design to achieve an active IND
Project Team & Resources	Evaluate the expertise and resources that will be deployed to achieve the project deliverables
Population Impact	Evaluate the extent to which the project considers the potential impact of the proposed therapy across affected populations

Pre-submission Components and Considerations

Overview of PDEV Application Process

Today's webinar will focus on pre-submission process



PDEV | Pre-submission Process Workflow

Pre-submissions open now

Pre-submissions due June 12

Applications open July 17

Applications due August 28

1 Pre-submission

Applicant completes a short pre-submission form in GMS

2 CIRM Reviews

CIRM filters & rank orders pre-submissions based on preferences and related objective criteria

3 Full Application

PDEV program invites select applicants to submit full application

Pre-submission Evaluation Criteria

Proposition 14 Preferences

- Pluripotent stem cell-derived therapies
- In vivo genetic therapies
- Diseases of the brain and CNS

Other Preferences

- Non-viral nucleic acid delivery
- Progression from DISC2 and TRAN1 awards
- Pre-IND or INTERACT meeting conducted

Under-represented therapeutic / disease area

- Targeting a therapeutic / disease area under-represented in CIRM active awards portfolio

Novelty of therapeutic approach

- Differentiation compared to CIRM active awards portfolio

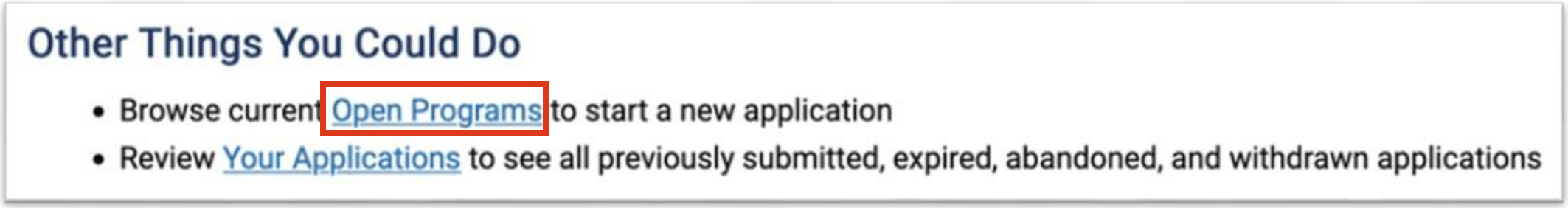
Pre-submission Process for PDEV

Pre-submission Forms

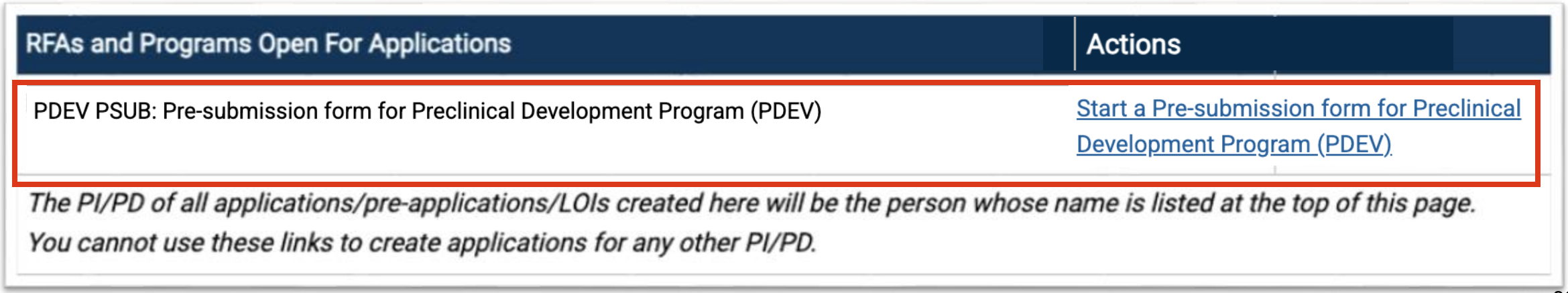
- Online pre-submission forms available to prospective applicants in the CIRM Grants Management System
- Forms will collect required information to perform initial eligibility and evaluate for program fit including:
  - Eligibility
  - Team Personnel
  - Project Title & Keywords
  - Project Information
  - Proposed Activities

Pre-submission: Getting Started

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



- Select “Start a Pre-submission form for Preclinical Development Program”



Pre-submission: Components

ONLINE SECTION

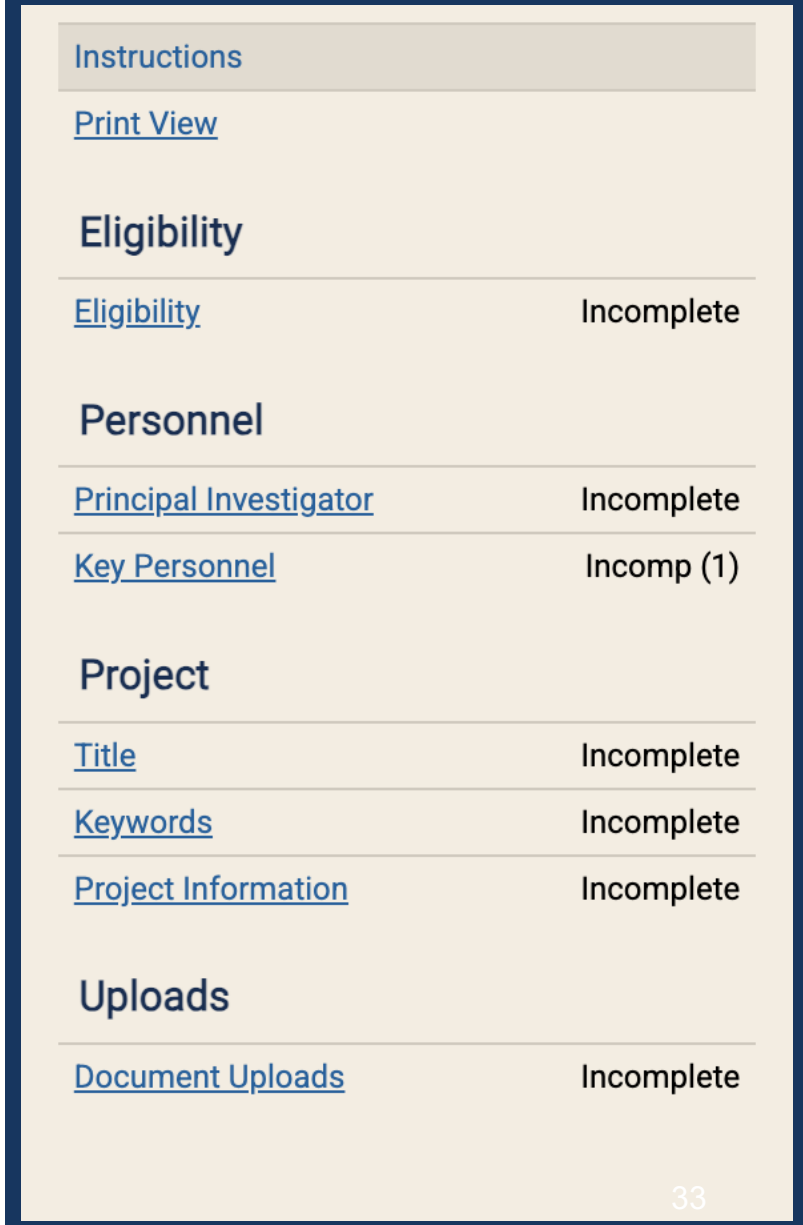
- Eligibility
- Personnel
- Project

UPLOADS SECTION (Attachment)

Required– must use templates provided

- Candidate Eligibility
- Disease Modifying Activity (DMA)

Required sections will change from "Incomplete" to "Complete" as you work through the pre-submission sections



Q&A

Common questions

Can I schedule a consultation for my pre-submission?

Consultation calls will not be conducted during pre-submission stage. Applicants who are invited to submit a full application will have the opportunity to request in-depth consultations with CIRM Science Officers.

Can non-CA investigators be included in PDEV application?

Non-CA based investigators are not eligible to serve as PI on PDEV awards. PDEV applicants are allowed to budget grant funds to support non-CA-based collaborators through a grant subcontract, provided the out-of-state organization DOES NOT retain the intellectual property or independent publication rights of any intellectual property arising out of the CIRM-funded project.

Do I have to show plans for Late PDEV activities when I'm at Early PDEV?

Yes, all PDEV applications will need to show plans to achieve IND clearance, which includes Late PDEV activities (i.e., IND-enabling).

Please visit our program page for more information

Today's webinar and final FAQ will be on our website in 2 weeks

https://www.cirm.ca.gov/preclinical/