

Access & Affordability (A&A) Strategy and Implementation Plan

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Agenda

1 Meeting Goal

2 Background & Objectives

3 Implementation Plan

4 Strategy Milestones

5 AAWG Recommendation

Purpose for today's meeting

Present the Access & Affordability
(A&A) Strategy to the AAWG for input
and recommendation to the ICOC

November AAWG
(Strategy recommendation)



December ICOC
(Strategy approval)

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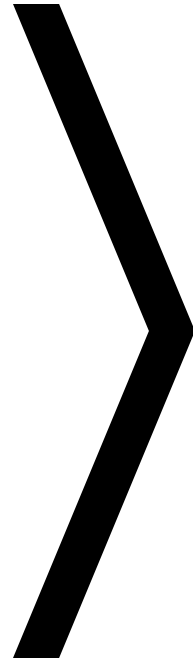
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Prop 14's Access & Affordability

Enhance **patient access**

Promote **affordability**



CIRM's Access & Affordability (A&A) Strategy provides a framework, integrated into existing programs and operations, to advance equitable access to CGT clinical trials and approved therapies for Californians

A&A Strategy Objectives

- 1. Clinical Infrastructure Integration**
- 2. Policy & Payer Engagement**
- 3. Affordability & Financial Innovation**

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1. Clinical Infrastructure Integration

Goal

Create a cohesive, performance-driven network that drives geographically distributed access of CIRM-funded clinical trials and therapies, making resulting treatments and cures broadly accessible to California patients

Key Components & Actions

- Alpha Clinics
- Community Care Centers of Excellence
- Patient Support Program
- Community-Based Organization Expansion Program

1. Clinical Infrastructure Integration

Key Components	Actions
Alpha Clinics (ACs)	• Enhance Operational Performance of Trials • Standardized Metrics
Community Care Centers of Excellence (CCCEs)	• Partnership with ACs • Geographic Expansion • Early-phase Readiness • Standardized Metrics
Patient Support Program (PSP)	• Integration with ACs/CLIN2 • Ancillary Support • Outcome & Equity Tracking
Community-Based Organization (CBO) Expansion Program	• Gap Analysis & Mapping • Pilot Program • Metrics & Accountability • CBO Coordination

1. Clinical Infrastructure Integration

Expected Outcomes:

- YoY increases in participation of underserved populations
- 100% of ACs/CCCEs reporting standardized metrics annually by FY27/28
- Statewide map of access points for CGT clinical trials and therapies
- Expanded geographic coverage aligned with population distribution
- Consistent statewide outreach practices across infrastructure and CBOs

2. Policy & Payer Engagement

Goal

Position CIRM as a credible and trusted convener among payers and policymakers to advance coverage and reimbursement models to ensure equitable access to CGTs to all Californians

Key Components & Actions

- Structured Payer Engagement Framework
- California CGT Reimbursement Pilot
- Policy Advocacy

2. Policy & Payer Engagement

Key Components	Actions
Structure Payer Engagement Framework	• Strategic Stakeholder Engagement • Technical Evidence Exchange Sessions • Foundation for Reimbursement Pilots
California CGT Reimbursement Pilot	• Public Payer Partnership • Scalability & Evidence Generation
Policy Advocacy	• Newborn & Early-Life Screening Linkages • Equity Lens • Partnership with Other State Agencies

2. Policy & Payer Engagement

Expected Outcomes:

- Establish engagement channels with a public payer and 2+ major commercial payers by FY28/29
- Support the launch of a California CGT reimbursement pilot by FY34/35
- Early diagnosis and screening pathways integrated into trial enrollment for rare diseases
- Demonstrable reduction in access disparities linked to socioeconomic barriers

3. Affordability & Financial Innovation

Goal

Promote that high-cost, potentially curative CGTs are accessible to all eligible Californians by embedding accessibility planning early in CIRM's R&D programs and advancing innovative financial models that make these therapies sustainable

Key Components & Actions

- Formalizing A&A Planning in Awards
- Financial Modelling & Innovation
- Rare Disease Commercialization and Financial Innovation Program

3. Affordability & Financial Innovation

Key Components	Actions
Formalizing A&A Planning in Awards	• Embedded Milestones
Financial Modeling & Innovation	• Financial Model Development • Portfolio Integration • Cost Reduction Through Manufacturing Innovation
Rare Disease Commercialization & Financial Innovation Program	• New Models for Sustainable Delivery and Access

3. Affordability & Financial Innovation

Expected Outcomes:

- 100% of CLIN2 and PDEV awardees integrate A&A Toolkit–based reimbursement and affordability milestones by FY 26/27
- Implementation of at least one California-based financial pilot by FY31/32
- Sustained affordability frameworks adopted by Medi-Cal and commercial payers for CGTs originated from CIRM's pipeline

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Near-Term Milestones – by FY26/27

Operational

- Finalize internal A&A strategy and governance structure

Objective 1 - Clinical Infrastructure Integration

- Establish standardized metrics to track ACs/CCCEs performance
- Expand reach to under-represented populations by successfully onboarding and developing community-based strategies for engagement, referral and enrollment

Objective 2 - Policy & Payer Engagement

- Initiate payer engagement framework and first knowledge-exchange session

Objective 3 - Affordability & Financial Innovation

- Engage external financial modeling experts and deliver recommendations

Long-Term Milestones – by FY34/35

Objective 1 - Clinical Infrastructure Integration

- Demonstrate measurable increases in trial participation of underrepresented populations across all ACs/CCCEs.

Objective 2 - Policy & Payer Engagement

- Support the initiation of a functioning California CGT reimbursement pilot with a public payer and at least two commercial payers
- Achieve 100% of BLA-ready therapies with access and reimbursement plans

Objective 3 - Affordability & Financial Innovation

- Operationalize a sustainable commercialization model for at least one rare disease therapy

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Recommendation

CIRM requests a recommendation from the AAWG to advance the draft Access & Affordability Strategy to the ICOC for approval