CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
ACCESS & AFFORDABILITY (A&A) STRATEGY & IMPLEMENTATION PLAN

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I. Executive Summary

CIRM's Access & Affordability (A&A) Strategy provides a framework, integrated into existing programs and operations, to advance equitable access to cell and gene therapy (CGT) clinical trials and approved therapies for Californians. Grounded on the mandates of Proposition 14 to **enhance patient access** and **promote affordability of CIRM funded therapies**, the A&A strategy integrates infrastructure, policy, payer engagement, and financial innovation into a cohesive plan with measurable goals for patient participation, cost reduction, and geographic availability.

II. Objectives

CIRM's Access & Affordability Strategy is designed to turn Proposition 14's mandates into measurable action through interconnected objectives, reflecting the need to strengthen infrastructure, provide access, ensure affordability, shape policy, and demonstrate accountability.

1. **Clinical Infrastructure Integration:** *Expand patient access to CIRM-supported therapies* for all eligible Californians by building an integrated infrastructure (Alpha Clinics, CCCEs, PSP, and community partnerships) that accelerates trial readiness, removes geographic and socioeconomic barriers, and drives equitable enrollment.

[1] Detailed initiative prioritization and feasibility scoring for this pillar are included in Appendix A.

2. **Policy & Payer Engagement:** *Advance coverage and reimbursement pathways* by building structured engagement with public and private payers to inform evidence needs, strengthen payer confidence in CIRM-supported therapies, and identify opportunities for innovative payment models and policy solutions that support long-term patient access.
3. **Affordability & Financial Innovation:** *Improve affordability and economic sustainability* by embedding early planning requirements into R&D programs to identify cost drivers, generate payer-relevant evidence, and explore financial mechanisms that help awardees develop viable strategies for reducing patient out-of-pocket costs and supporting long-term therapy access.

III. Implementation Plan [(1)]

1. Clinical Infrastructure Integration

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

Rationale: To translate scientific advances into real patient impact, CIRM must move beyond standalone programs and build an integrated infrastructure that functions as a unified, statewide system. Prop14 mandates expansion of clinical infrastructure to make therapies broadly available through geographically distributed centers and patient support programs. Achieving this requires alignment across Alpha Clinics (ACs), Community Care Centers for Excellence (CCCEs), the Patient Support Program (PSP), and patient navigation through Community Based Organizations (CBOs) to reduce startup delays, eliminate geographic disparities, and create a sustainable pathway for delivering advanced therapies to all California patients, not just those near major academic centers.

Key Components & Actions:

1. **Alpha Clinics:**
    - **Enhance Operational Performance of CIRM-funded trials:** Promote coordination between CLIN2 awardees and ACs to reduce operational bottlenecks for rapid enrollment of California patients.
    - **Deploy Standardized Metrics across ACs:** Expand and implement uniform reporting for trial volume, enrollment diversity, retention, socioeconomic/geographic reach to evaluate the impact of the ACs and adapt CIRM's A&A programs to better serve California patients.
2. **Community Care Centers of Excellence (CCCEs)**
    - **Partnership with ACs:** Establish structured referral standards and data-sharing across CCCEs and ACs to create coordinated patient pipelines.
    - **Geographic Expansion:** Expand geographic reach of program through future funding.
    - **Early-Phase Readiness:** Build capacity at CCCEs to support IND-enabling and first-in-human studies.
    - **Enhanced Navigation & Social Support:** Integrate navigation services that address social determinants (ex., transportation, housing instability, language, digital literacy, childcare, cultural trust) to reduce barriers to participation and improve representation of populations historically excluded from clinical research.

[(1)] Detailed initiative prioritization and feasibility scoring for this pillar are included in Appendix A.

- **Deploy Standardized Metrics across CCCEs:** Implement uniform reporting for trial volume, enrollment diversity, retention, socioeconomic/geographic reach to evaluate the impact of the CCCEs and adapt CIRM's A&A programs to better serve California patients.

3. **Patient Support Program (PSP):**
    - **Integration with ACs/CLIN2:** Integrate with ACs and CLIN2s
    - **Ancillary Support:** Expand to cover additional clinical trial costs (ex. childcare, and related services) to reduce barriers for underserved patients.
    - **Outcome & Equity Tracking:** Measure and track patient referrals, demographics and services used to evaluate the impact of the PSP program and adapt to how best provide patient support services to California patient population.
4. **Community Based Organization (CBO) Expansion Program:**
    - **Gap Analysis & Mapping:** Compare CIRM trial participants with California's demographic distribution to identify underserved populations and geographies.
    - **Pilot Program:** Launch competitive awards for CBOs beyond CCCEs, prioritizing high-need regions identified in gap analysis.
    - **Metrics & Accountability:** Collect data on outreach, referrals, trial enrollment, and equity impacts.
    - **CBO coordination:** Align and coordinate funded CBOs; standardize educational and culturally relevant outreach materials.

Expected Outcomes:

- Year-over-year 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.

Rationale: Payers face uncertainty regarding the durability, cost, and value of CGTs, leading to uneven coverage decisions and access delays. As a steward of California's investment, CIRM plays an active role enabling therapies it funds to be delivered to patients. By leveraging its portfolio data and clinical infrastructure, CIRM can inform policy development, facilitate payer engagement, and contribute data driven input in the development of reimbursement models that promote timely and equitable access.

Key Components & Actions:

(1) Detailed initiative prioritization and feasibility scoring for this pillar are included in Appendix A.

1. **Structured Payer Engagement Framework**
    - **Strategic Stakeholder Engagement:**
      Engage with public payers, commercial plans, self-insured employers, and provider networks focused on shared challenges (between both payers and therapy developers) such as durability evidence, long-term cost modeling, and budget impact, using CIRM's clinical portfolio and infrastructure as the data backbone.
    - **Technical Evidence Exchange Sessions:**
      Convene data-driven working sessions that bring together CIRM awardees, providers, and payers to review real-world evidence, durability signals, and patient outcomes, with the goal of informing affordability planning, identifying evidence gaps, and building payer confidence in forthcoming therapies.
    - **Foundation for Reimbursement Pilots:**
      Use insights from these engagements to shape and support initiation of targeted California-based reimbursement pilots tied to specific late-stage CIRM-funded therapies.
2. **California CGT Reimbursement Pilot**
    - **Public Payer Partnership:** Collaborate with public payers to help inform the development of pilot reimbursement models that distribute financial risk between payers and therapy developers, informed by outcomes and durability data from CIRM's clinical portfolio.
    - **Scalability & Evidence Generation:** Use aggregated durability and outcomes data from CIRM's portfolio to validate payment triggers and expand model to commercial payers.
3. **Policy Advocacy**
    - **Newborn & Early-Life Screening Linkages:** Advocate/promote for state policies that link newborn and early life genetic screening with Alpha Clinics and CCCEs for rapid enrollment into rare disease programs.
    - **Equity Lens:** Align screening and access efforts with Prop 14's focus on equity and geographic distribution.
    - **Partnership with other State agencies:** Coordinate with California DHCS and Covered California to ensure coverage models for CGTs and financial support mechanisms.

Expected Outcomes:

- Established recurring payer engagement channels with public payers and at least two major commercial payers in FY 28/29.
- Support the launch of a California CGT reimbursement pilot by public payer by FY34/35.
- Early diagnosis and screening pathways integrated into trial enrollment for rare diseases.

(1) Detailed initiative prioritization and feasibility scoring for this pillar are included in Appendix A.

- 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. CIRM will leverage its funding and convening power to require, test, and scale structured affordability planning frameworks, from project milestones to statewide pilots, that de-risk access and uphold California's commitment to equitable innovation.

Rationale:
Even when therapies prove scientifically successful, affordability remains one of the greatest barriers to patient access. Even the most transformative therapies can fail to reach patients due to inadequate reimbursement, lack of payer confidence, and unaffordable costs.

CIRM has recently implemented the foundational A&A Toolkit and is establishing baseline expectations for A&A planning within awardee programs. CIRM must now move beyond this foundation and build the broader system required to operationalize it. This includes considering scaling financial model development, embedding reimbursement and affordability metrics into portfolio management, and tackling upstream cost drivers such as manufacturing and commercialization inefficiencies. Together, these actions will promote that every therapy supported by CIRM is not only scientifically sound, but also economically viable, equitably accessible, and sustainably deliverable to California patients in the real world.

Key Components & Actions:

1. **Formalizing A&A planning as part of Awards**
    - **Embedded Milestones:** Every CLIN2 and PDEV project is now required to demonstrate early payer awareness and affordability planning through stage-appropriate milestones, informed by the A&A Toolkit.
2. **Financial Modeling & Innovation**
    - **Financial Model Development:**
      Engage with financial professionals, reimbursement experts and policy advisors to develop novel financial models and assess their real-world feasibility for supporting affordability of CGTs.
    - **Portfolio Integration:**
      Findings from these modeling efforts will be incorporated into future funding opportunities and CIRM's programmatic expectations.
    - **Cost Reduction Through Manufacturing Innovation**
      Leverage CIRM funding programs to support innovative manufacturing technologies and operational models that streamline CMC regulatory requirements and lower costs-of-goods for CGTs
3. **Rare Disease Commercialization & Financial Innovation Program**
    - For ultra-rare or low-commercial-value therapies, CIRM will explore new models for sustainable delivery and access (please refer to the prioritization table in Appendix A for some possible models)

(1) Detailed initiative prioritization and feasibility scoring for this pillar are included in Appendix A.

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 (ex., risk-pooling or bond mechanism) by FY31/32
- Sustained affordability frameworks adopted by Medi-Cal and commercial payers for CGTs originated from CIRM's pipeline.

IV. Cross-Cutting Enablers

- **Dedicated A&A Leadership:** Establish a senior-level owner with cross-functional authority to steward this strategy.
- **Data & Analytics Infrastructure:** Create a unified A&A dashboard integrating trial metrics and diversity data.
- **Governance:** Use AAWG as the formal body to review pilots, advise on policy, approve initiatives and recommend course corrections.
- **Gap Analysis & Mapping**
  As a formal enabling activity (Q4 FY25–Q2 FY26), CIRM will conduct a comprehensive gap analysis and mapping exercise. This will:
  - Identify underserved geographies and populations relative to CIRM trial participation
  - Create a baseline "CIRM Trial Coverage Map"
  - Inform prioritization of CBO co-funding pilots and long-term expansion
  - This foundational activity will anchor the Patient Engagement & CBO Expansion Program and guide resource allocation across the A&A strategy

V. Near-Term Implementation Timeline (FY25/26–FY26/27)

- Finalize internal A&A strategy and governance structure (Q4 FY25/26).
- Establish standardized metrics to track ACs/CCCEs performance (Q1 FY26/27).
- Initiate payer engagement framework and first knowledge-exchange session (Q2 FY26/27).
- Engage external financial modeling experts and deliver recommendations (Q2 FY26/27).
- Expand reach to under-represented populations by successfully onboarding and developing community-based strategies for engagement, referral and enrollment.

VI. Long-Term Milestones (By FY34/35)

(1) Detailed initiative prioritization and feasibility scoring for this pillar are included in Appendix A.

- Demonstrate measurable increases in trial participation of underrepresented populations across all ACs/CCCEs.
- Establish 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.
- Operationalize a sustainable commercialization model for at least one rare disease therapy.

VII. Appendix A: Initiative Prioritization Tables

1. Clinical Infrastructure Integration

Initiative	Status	Description	Impact & Alignment	Feasibility (Budget/ Operational/Time)	Impact & Alignment Score
Alpha Clinics	In Progress	Standardize reporting across sites (trial volume, diversity, catchment, start-up times).	Direct support for pipeline; reduces start-up delays.	High / High / High	6
CCCEs	In Progress	Integrate with Alpha Clinics via referrals, SOPs, and early-phase readiness.	Expands reach into underserved regions; builds trial capacity.	High / High / High	8
Patient Support Program	In Progress	Develop single-entry triage; expand ancillary supports (housing, childcare, travel).	Supports patient navigation, retention, equity.	High / Medium / High	7
CBO Expansion	Not Started	Expand outreach beyond CCCEs; launch competitive CBO pilot program.	Directly addresses SAF Goal 5 on access and equity.	High / High / Medium	8

(1) Detailed initiative prioritization and feasibility scoring for this pillar are included in Appendix A.

2. Policy & Payer Engagement

Initiative	Status	Description	Impact & Alignment	Feasibility (Budget/ Operational/Time)	Impact & Alignment Score
Structured Payer Engagement Framework	In Progress	Relationships with Medi-Cal, commercial payers, and employers; pipeline briefings; capture payer insights; knowledge exchange.	Critical enabler of reimbursement readiness.	High / Medium / High	9
California-Specific Reimbursement Pilot	Not Started	Collaborate with Medi-Cal to develop risk-sharing model; aggregate outcomes; scalable to commercial payers.	Strong CGT and affordability alignment.	High / Medium / Medium	9
Early Diagnosis Pathways / Policy Advocacy	Not Started	Advocate linkage of rapid screening/diagnosis to ACs and CCCEs; educate payers on rare disease enrollment.	Supports SAF rare disease pipeline and Goal 5.	High / High / Medium	9

3. Affordability & Financial Innovation Initiatives

Initiative	Status	Description	Impact & Alignment	Feasibility (Budget/ Operational/Time)	Impact & Alignment Score
Reimbursement Readiness Checklist	In Progress	Embed checklist in CLIN2/PDEV awards to ensure payer alignment early.	Supports affordability and preparedness.	High / Medium / Medium	8
Novel Financing Models	Not Started	Explore outcome-based agreements, stop-loss funds, pooled risk models.	Needed to de-risk adoption of high-cost therapies.	Medium / High / High	9

(1) Detailed initiative prioritization and feasibility scoring for this pillar are included in Appendix A.

Initiative	Status	Description	Impact & Alignment	Feasibility (Budget/ Operational/Time)	Impact & Alignment Score
Manufacturing Cost Reduction	Not Started	Fund automation, allogeneic approaches, point-of-care models to lower COGs.	Critical to affordability and therapy sustainability.	High / High / Medium	8
Rare Disease Commercializati on / CLINX Pilot	Not Started	Aggregator model for ultra-rare diseases; explore public-benefit and treatment IND pathways.	Supports SAF rare disease strategy; highly innovative.	High / Medium / Medium	6

Note: Detailed initiative prioritization and feasibility scoring are provided here as an appendix. Each pillar section in the main document makes reference to these tables for operational detail and prioritization rationale.

How to Interpret the Initiative Prioritization Tables

Each initiative in the following tables was evaluated using a standardized framework to guide sequencing, resource allocation, and implementation timing within CIRM's Access & Affordability (A&A) Strategy. Scores and classifications reflect alignment with Prop 14 mandates, SAF goals, and organizational capacity to execute.

Field	What It Means
Status	Indicates current progress: *In Progress* (active), *Not Started* (planned), or *Completed* (implemented).
Impact & Alignment	Qualitative assessment of how directly the initiative fulfills Proposition 14 statutory requirements (e.g., affordability, equitable access, statewide infrastructure) and its potential impact.
Feasibility Score (High/Medium/Low)	Composite rating of *budget need, operational complexity,* and *time to implement.* Used to determine sequencing and near-term focus.
Budget Feasibility	High – Minimal investment required for R&D / infrastructure; Uses existing projected program funds that have already been allocated.

(1) Detailed initiative prioritization and feasibility scoring for this pillar are included in Appendix A.

Field	What It Means
	Medium – Moderate investment required for R&D / infrastructure; Potential for funds to be allocated.
	Low – High investment required for R&D / infrastructure; Little to no potential for funds to allocated.
Operational Feasibility	High – Minimal operating costs; Easily absorbed into current budget without major trade-offs.
	Medium – Moderate operating costs; Requires budget adjustments but is sustainable.
	Low – High operating costs; Requires major budget reallocation or new funding.
Time to Implement	High – Measure of impact will be effectual within 2-3 years of implementation.
	Medium – Measure of impact will be effectual within 6-10 years of implementation.
	Low – Measure of impact will be effectual in >10 years after implementation.
Impact & Alignment Score	Combines Impact & Mission Alignment scores to rank initiatives: **High Priority (≥8)** – aligned with P-14 and high-impact; **Medium (6–7)** – moderate alignment and impact; **Low (≤5)** – low impact and alignment with P-14.

VIII. Appendix B: Definitions for Access and Affordability Implementation

For the purposes of the CIRM Access and Affordability (A&A) Strategy and Implementation Plan, the following terms are defined as follows:

Programmatic Activities

- Patient Outreach – Activities that provide general information about CIRM programs and resources, including but not limited to the CIRM Clinical Infrastructure.
- Patient Engagement – Interactions with a patient or their representative to share information and identify CIRM Clinical Infrastructure resources that may address their medical needs.

(1) Detailed initiative prioritization and feasibility scoring for this pillar are included in Appendix A.

- Patient Navigation – Active support provided to help patients access and utilize CIRM Clinical Infrastructure resources. This may include referral to specific clinical trials or other relevant services.

Program Performance Metrics

- Patient Referral – An objective quantitative measure of impact where a patient's interaction with CIRM Clinical Infrastructure program or resources can be attributed to a specific education, engagement, or navigation activity.

- Patient Enrollment – An objective measure of impact reflecting the number of patients who have received at least one dose of an investigational product or approved therapy under a clinical protocol.

Note: The terms education and engagement are also used by CIRM and its awardees in other contexts. For example, CIRM conducts broad community engagement activities to educate Californians about agency programs. This appendix specifically defines terminology used for activities and metrics that support implementation of the Access and Affordability Strategy under Strategic Action Framework (SAF) Goal 5.

(1) Detailed initiative prioritization and feasibility scoring for this pillar are included in Appendix A.