

Real Life™

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Introduction to Proposed Roadmap on Access and Affordability

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CIRM
CALIFORNIA'S STEM CELL AGENCY

- Why we are creating a Roadmap to Access and Affordability
- Review the problem statement and the opportunity areas to address barriers to access and affordability
- Obtain ICOC feedback on proposed recommendations for consideration in the development of the Roadmap to Access and Affordability

Why are we Creating a Roadmap to Access and Affordability?

Prop 14 and The 5-year strategic plan state that CIRM will support the development of a roadmap for access and affordability in coordination with CIRM's AAWG.

The roadmap will include a strategy for gathering data to support reimbursement, engaging policymakers and regulators, and developing novel healthcare delivery models.

It will also manage future initiatives involving post-marketing research, follow-up and outcome database, registry development, real-world evidence research, healthcare economics, and patient-reported outcomes.

Problem Statement: There are several logistical and financial barriers to clinical trials and approved therapies for Cell and Gene Therapies



Opportunity: Prop14 provides dedicated funding and the formation of the Access and Affordability Working Group (AAWG) to address these barriers



Goal: The goal of the Roadmap is to identify near- and long-term initiatives to help patients overcome barriers to access and affordability



Based on research, communications with sites and analysis of the current landscape, these areas can serve to address and mitigate the barriers to Access and Affordability for patients



INITIATIVES THAT MITIGATE NEGATIVE SOCIAL AND CULTURAL DETERMINANTS OF HEALTH

- Expansion of Alpha Clinics
- Community Outreach and Education
- Future Community Care Centers of Excellence (CCCE)
- Community Partners

PROGRAMS TO ADDRESS INFORMATIONAL BARRIERS

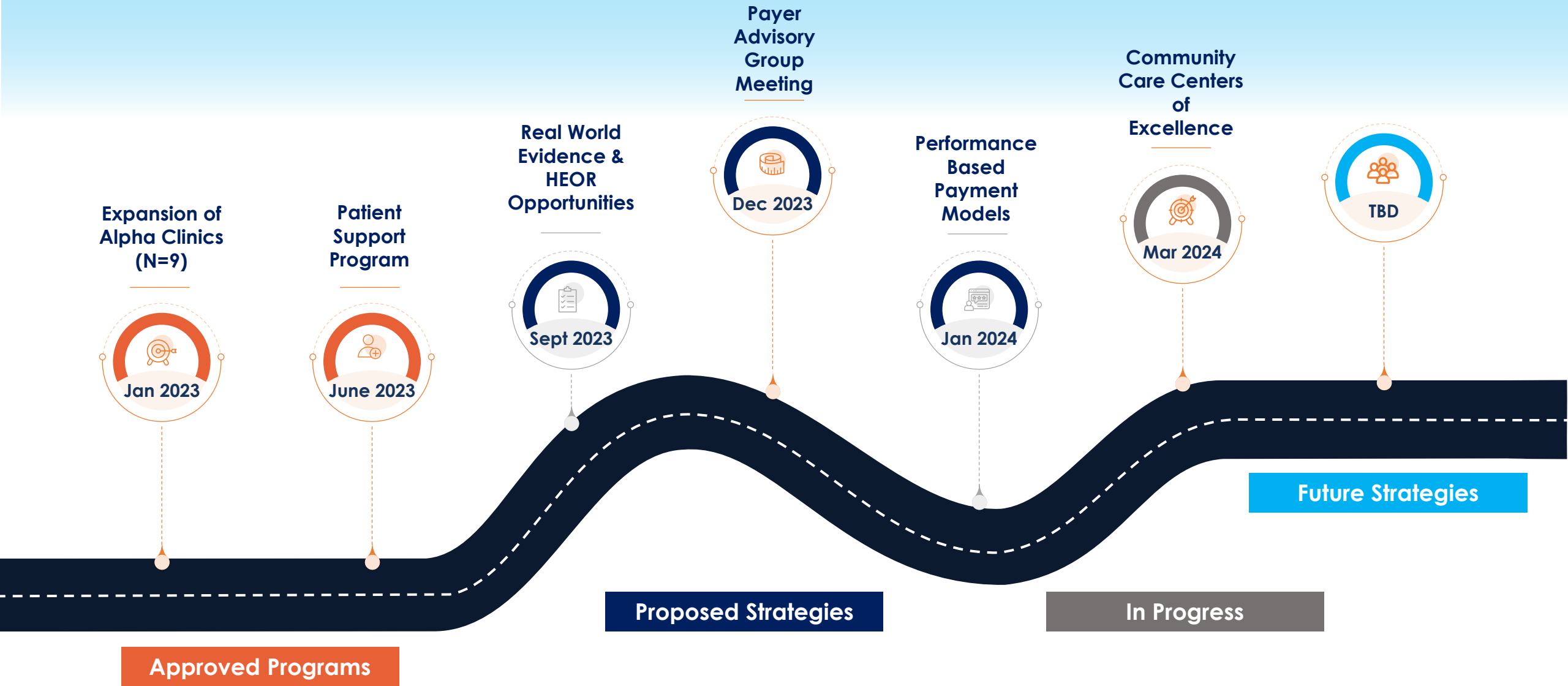
- Patient Support Program
- Integration with Alpha Clinics, CCCE, and Community Partners

PROGRAMS TO HELP PATIENTS NAVIGATE LOGISTICAL BARRIERS

- Patient Support Program
- Patient Assistance Fund
- Patient Navigators at the community level
- Stem Cell Counselors

SUPPORT BROAD AND FAIR COVERAGE FOR CELL AND GENE THERAPIES

- Research Real World Evidence and Health Outcomes Opportunities
- Engagement with Payers to Incentivize Broad Access and Transparent Coverage Policy
- Evaluate Outcomes-Based Payment Models



Prop 14: Promote Access to Trials and Create Novel Healthcare Delivery Models



ALPHA CLINIC EXPANSION

Activity

Ongoing support for 9 Alpha Clinics; Progress reports from sites to provide new learning and opportunities to expand access and affordability for CGT

Funding for Alpha Clinics is a key element to CIRM's strategy to reduce barriers to access clinical trials of CGT for all Californians

Prop 14: Offsetting the Costs of Providing Treatments and Cures Arising from Institute Funded Research



PATIENT SUPPORT PROGRAM (PSP)

Activity

Create consistent, efficient, streamlined, and reliable access to resources and support across all the different types of CIRM-funded programs

Patient participation in CGT trials is complex, costly, and demanding, such that many Californians and their families require considerable logistical and financial support, particularly underserved patient populations

Prop 14: Enhance the Geographical Distribution to Promote Patient Access to Trials and Create Novel Healthcare Delivery Models



COMMUNITY CARE CENTERS OF EXCELLENCE (CCCE)

CCCE's are geographically diverse centers of excellence that promote access to clinical trials and potential treatments arising from CIRM-funded research

CCCE's are qualified sites with clinical, operational, and educational expertise to provide rural communities' access to investigational cell and gene therapy trials, access to CIRM-funded commercial therapies, and robust educational efforts for regenerative therapies

Activity

Needs Assessment:

Listening sessions and public workshop

Board Review:

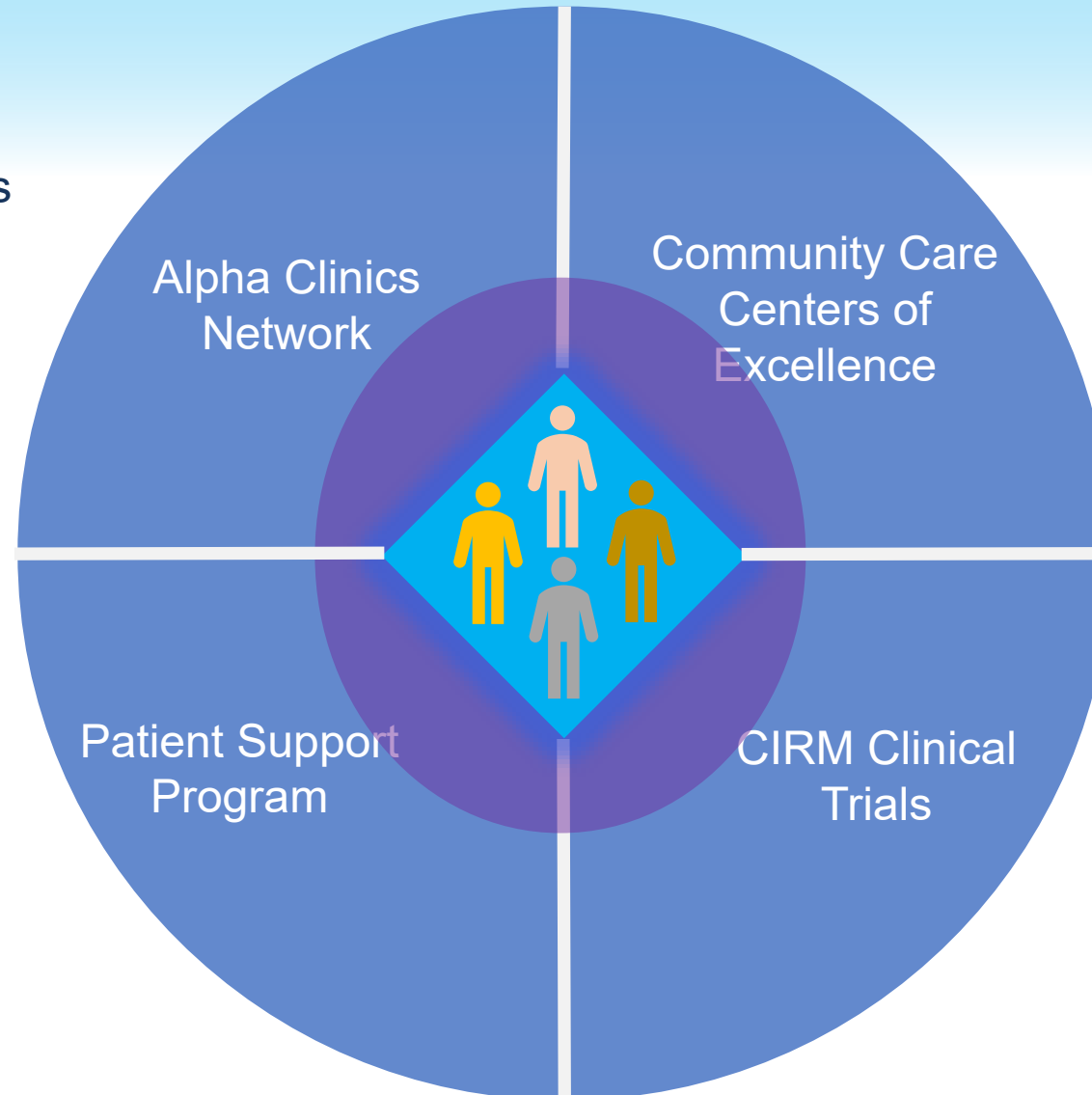
AAWG, SS, ICOC review of concept proposals in 2023

Application Phase:

RFAs initiated in Early 2024

- Nine Lead Medical Centers
- Supporting CIRM-Funded Clinical Trials

- Address Financial and Logistical Barriers
- Improve Patient and Research Outcomes



- Bring Clinical Capacity to Communities
- Address Social Determinants Impacting Participation

- 91 CIRM-Funded Clinical Trials
- Optimal Care for Unmet Medical Needs

Proposed Strategies to Consider

Support Funding of CA Disease Registries and Other Real-World Data Collection



Benefit: Enhances opportunity for approval and payer reimbursement when data is appropriately substantiated according to FDA guidance and payer mandates. Patients will have access to the treatment if approved and reimbursed by payers

Activity	Why this Matters?
<p>Real-world studies using data from administrative databases, electronic medical records and medical charts, patient surveys, registries</p>	<ul style="list-style-type: none"> • Natural history study to generate an appropriate control for rare disorders • Support submissions to FDA for marketing authorization and to satisfy post marketing requirements • Submit as evidence of long-term outcomes (beyond the clinical trial follow-up period) to private and public payers

Payer Engagement



Benefit: Creates proactive understanding of payer issues, necessary data needed for appropriate payment models

Potential Activities	Why this Matters?
<p>Engagement with payers via advisory boards, 1:1 interviews, and surveys (one-time or periodic)</p>	<ul style="list-style-type: none"> • Prioritization of evidence generation for CGT; patient-reported outcome measures and other endpoints relevant for coverage • Identify key elements and inputs to performance-based payment models • Articulate payer concerns, anticipate CGT coverage policies and address potential limitations on access • Identify Payer Access and Affordability initiatives

Research to Address State Policy Issues



Benefit: Develop an understanding of the state policy to support proactive communication and development of CIRM policies with key California agencies

Potential Activities	Why this Matters?
<p>Qualitative policy research; Engagement with CMS and other agencies</p>	<p>Proposed research topics:</p> <ul style="list-style-type: none"> • Research and formulate CIRM position on CMS Cell and Gene Therapy Access Model, a potential financing approach for patients enrolled in Medicaid • Research the implications of new or planned legislation, including the Cancer Care Equity Act • Policy research and coordination with related agencies, including the CA Office of Health and Affordability

Approved Timeline

Expansion of Alpha Clinics (6 – 9 Clinics)



Jan 2023

Patient Support Program



RFA is Posted

In Progress Timeline

Community Care Centers of Excellence

March 2024

Possible Strategies Timeline

RWE Opportunities



September 2023

Payer Advisory Group Meeting



December 2023

Performance-Based Payment Models



January 2024

Future Concepts Timeline

- Are the proposed research activities appropriate as initial steps of implementation?
- What can we consider as longer-term initiatives?

Thank you