

C I R M

CALIFORNIA INSTITUTE FOR
REGENERATIVE MEDICINE

Access & Affordability Working Group

Shyam Patel, PhD

Geoffrey Lomax, Dr.PH

May 8, 2026

Agenda

- ① **ISSCR Access & Affordability Summit**
- ② **FDA Workshop: Advancing Pediatric CGT Trials**
- ③ **Rocket LAD-1 Therapy Approval**
- ④ **Updates to Access Plan Requirements for Preclinical and Clinical Programs**
- ⑤ **CCCE Updates**

ISSCR Summit on Access & Affordability in Cell & Gene Therapies

March 20, 2026, UCLA

- Broad Stakeholder representation across academia, industry, payers and government
- Timely & relevant themes
 - Payer Participation
 - Emerging Policy and Affordability Models
 - Manufacturing & Regulatory Innovation
- CIRM Awardees & AAWG Members well represented
- Co-organized by Maria Bonneville

ISSCR Access & Affordability Summit

Key Takeaways

1. Current US healthcare delivery, insurance and payment systems not well aligned with curative or one-time therapies— systems must evolve
2. Therapy developers must plan early for patient and market access for CGTs
3. Manufacturing innovation is essential to long-term affordability and access

Agenda

- ① ISSCR Access & Affordability Summit
- ② **FDA Workshop: Advancing Pediatric CGT Trials**
- ③ Rocket LAD-1 Therapy Approval
- ④ Updates to Access Plan Requirements for Preclinical and Clinical Programs
- ⑤ CCCE Updates

FDA Workshop: Advancing Pediatric CGT Clinical Trials

April 09, 2026, FDA Campus

- Challenges and solutions for testing and delivering CGTs to pediatric patient populations
 - Scientific, regulatory, ethical and practical considerations
- Perspectives from diverse stakeholders - FDA, clinicians, parents, patient advocates and industry
- Patient Access Considerations:
 - Enrolling pediatric patients in first-in-human trials
 - Eligibility and endpoints decisions that impact eventual real-world delivery
 - Presymptomatic/early disease treatment considerations
- Workshop co-sponsored by CIRM

Agenda

- ① ISSCR Access & Affordability Summit
- ② FDA Workshop: Advancing Pediatric CGT Trials
- ③ **Rocket LAD-1 Therapy Approval**
- ④ Updates to Access Plan Requirements for Preclinical and Clinical Programs
- ⑤ CCCE Updates

Rocket Pharma LAD-1 Therapy KRESLADI Receives FDA Approval

- First approved therapy for rare disease LAD-1
- First CIRM-funded therapy to reach BLA approval
- Product launch end of 2026
- Access plan will be reviewed by CIRM



Agenda

- ① ISSCR Access & Affordability Summit
- ② FDA Workshop: Advancing Pediatric CGT Trials
- ③ Rocket LAD-1 Therapy Approval
- ④ Updates to Access Plan Requirements for Preclinical and Clinical Programs**
- ⑤ CCCE Updates

Updates to Access Plan Requirements

For Preclinical and Clinical Programs

Board Feedback

1. Patient access is an important consideration in CIRM funding decisions
2. Affordability is complex and should not be factored into CIRM funding decisions
3. Applicants and Reviewers require clear and thorough guidance
4. Patient voice and expert input should be preserved in access evaluation

CIRM Updates

1. Reframed requirements as “Patient Access Strategy”
2. Access plans are stage-appropriate
3. Application instructions, review criteria, and reviewer training to be updated
4. Access specialists remain part of CLIN2 GWG review
5. Patient Advocate reviewers to have a more defined scoring role

Agenda

- ① ISSCR Access & Affordability Summit
- ② FDA Workshop: Advancing Pediatric CGT Trials
- ③ Rocket LAD-1 Therapy Approval
- ④ Updates to Access Plan Requirements for Preclinical and Clinical Programs
- ⑤ **CCCE Updates**

Awarded CCCE Sites

Launch Details

- Three awards launched **March 1, 2026**
 - ~\$9M per award
 - 5-year grants
- Orientation sessions with Alpha Clinic Program Directors kicked off mid-April
- Site visits to each of the awarded sites planned for May and June

Awarded CCCE Sites

- SoLA-CCERM (Torrance, CA)
- Inland Empire and Desert Region CCCE (Loma Linda, CA)
- Community Health System CCCE (Fresno, CA)



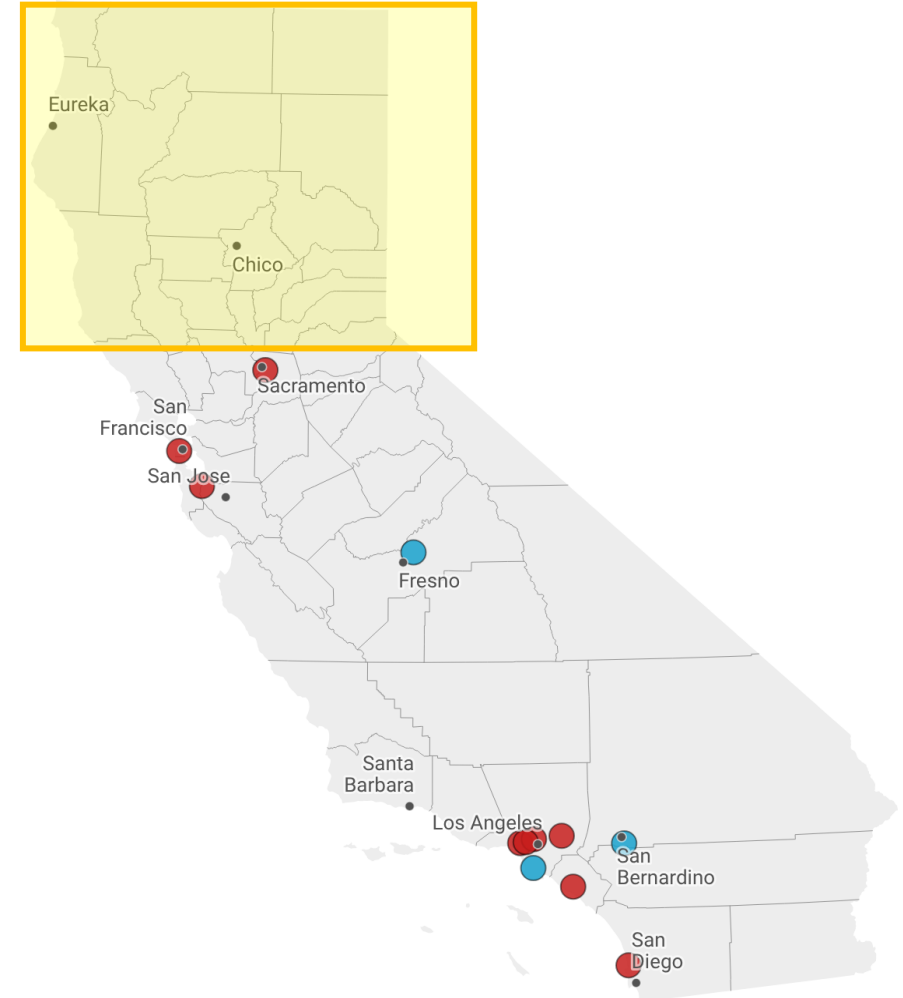
INFR8 RFA Re-Opening

Northern California CCCE

- In October 2025, ICOC expressed concerns that northern regions of CA not served by CIRM infrastructure
- In March 2026, ICOC voted to re-open the INFR8 RFA for applicants in designated counties
- Applications currently open until June 4

CIRM Clinical Infrastructure

- Alpha Clinic
- Community Care Center of Excellence



Thank you