

Real Life™

CIRM Access and Affordability Working Group
Coverage Analysis and Performance-Based Models
Sean Turbeville, PhD
May 02, 2023

CIRM
CALIFORNIA'S STEM CELL AGENCY

Today's Focus: Patient Expenses and Facilitating Reimbursement via Coverage Analysis and Performance-Based Models



DELIVER A ROADMAP FOR ACCESS AND AFFORDABILITY OF REGENERATIVE MEDICINE FOR ALL CALIFORNIA PATIENTS



Patient Support Services	Engagement with Payers	Creation and/or endorsement of new State policy for C>	Alpha Clinics and CCCE
Alpha Site Coordination and Alignment	Performance-Based Models	Treatment across state lines	Patient and post-marketing Registries, RWE, HEOR
Patient Navigator at CCCEs	Introduce New Payer Mechanisms for Orphan Products	Inpatient vs. Outpatient reimbursement	Accelerate Advocacy at community level; Medical Education
Coverage Analysis , Insurance Support, Co-Pay Assistance, Access and Appeals	Risk Pools/Coalition Partners	Align to Gov Plan on Office of Health and Affordability	State and Federal authorized treatment centers

Problem Statement: Lack of transparency of payers' coverage policies for Cell and Gene Therapies

Narrow coverage policies limit access to Cellular and Gene Therapies (CGTs)

- Many CGTs will reach the market in the next 3 years, many with high upfront costs [1-2]
- Payers design narrow coverage policies for CGT to reduce their financial risk [3-5]

Examples of restrictive coverage policies for CGT

- [Anthem coverage policy for Zolgensma](#) for spinal muscular atrophy [6]
- [BCBS MA coverage policy for Hemgenix](#) for Hemophilia B [7]
- [Credence BCBS coverage policy for Luxturna](#) for biallelic RPE65 mutation-associated retinal dystrophy [8]
- Aetna coverage policy for [tisagenlecleucel \(Kymriah\)](#), [tocilizumab \(Actemra\)](#), and [axicabtagene ciloleucel](#) [9-11]

Recommendation

CIRM plans to convene a **coverage analysis** of payer policies for CGT to review and advise on opportunities CIRM can deploy to help California patients.

Problem Statement: Lack of standards for performance-based payment models

- **Performance-based payment models** are contracts that tie reimbursement of CGTs to real-world data outcomes [1-3]
 - Annuity Based Payments
 - Outcomes-based Payments
 - Outcomes-based Annuity
 - Value-Based Warranties
- Operationalizing these innovative payment models requires: [3-5]
 - Collection of long-term health outcomes evidence
 - Contracts that make reimbursement contingent on demonstration of clinical benefit
 - Standards that reflect health outcomes relevant to patients specific for their condition

Recommendation

CIRM plans to research standards for **innovative performance-based payment models** to collect relevant and appropriate outcomes data, addressing operational challenges in data collection while maximizing patient access and affordability.

Roadmap Opportunities to improve Access and Affordability of CGTs

- **Coverage Analysis:** Increase transparency of coverage policies on CGTs
- **Performance-based payment models:** Research payment models and outcomes data to reduce complexity
- **Reinforces** the importance of **Real-World Evidence infrastructure** for Regulatory Authorities and Private/Public Payers

- Patient Support Services
- New Payer Models
- Inpatient to outpatient setting (e.g., CAR-T)
- State and Federal Policy (e.g., CCEA)
- Alpha Clinics and Community Care Centers of Excellence (CCCE)
- Real World Data, Health Economics Outcomes and Research (HEOR), Post-Marketing requirements
- Coverage Analysis and Insurance Restrictions
- **Next Presentation – 5-year Roadmap to Access and Affordability**

Thank you and Discussion