

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

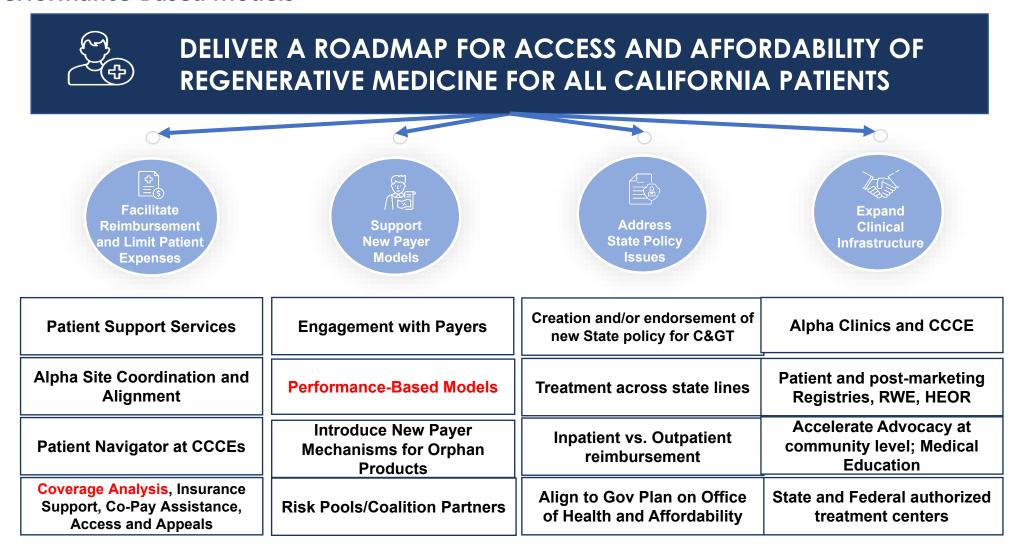




Roadmap to Access & Affordability Strategies



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







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 <u>tisagenlecleucel</u> (<u>Kymriah</u>), <u>tocilizumab</u> (<u>Actemra</u>), and <u>axicabtagene ciloleucel</u> [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.

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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



Summary of AAWG Presentations and Considerations for Roadmap



- 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