

Real Life™

CIRM Scientific and Medical Accountability
Standards Working Group
January 5, 2023 South San Francisco

CIRM
CALIFORNIA'S STEM CELL AGENCY

A new department within CIRM tasked with developing CIRM's Roadmap for Access and Affordability in coordination with the AAWG, and responsible for post-marketing research, outcomes, registries, real world evidence, health economics and policy.

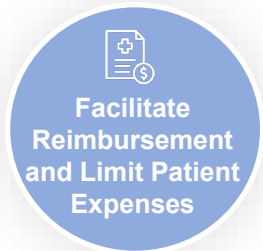
Five major workstreams:

- Alpha Clinics
- Access and Affordability Working Group
- Standards Working Group
- Community Care Centers of Excellence
- Patient Support Program



Roadmap to Access & Affordability

Strategies - Examples



Patient Support Services	Engagement with Payers	Creation and/or endorsement of new State policy for C&GT	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

What Barriers Must be Overcome to Achieve Broad, Equitable Access to Regenerative Medicines?



CULTURAL AND SOCIAL DETERMINANTS

- Lower enrollment for minorities [2]
- Socioeconomic status, unemployment, education [4,5]
- Population size/geography [6,7]
- Stigma of disease [8,9]



INFORMATIONAL

- Physician low referral rate [10]
- Medical mistrust and misinformation about regenerative medicine [11]



LOGISTICAL

- Lack reliable transportation [12,13]
- Language [14]
- Work or childcare requirements [11]



FINANCIAL

- Cost of regenerative medicines (gene or cell therapies) and insurance benefits may include high copays and lifetime benefit [15]



ABILITY-BASED

- Participation is limited for elderly, [16] adolescent and young adult, [17] and disabled patients [18,19]

Cell and gene therapy trials are becoming more demanding on patients and require additional support beyond routine costs for patients and their families

New Patient Assistance Programs have emerged to address bottlenecks associated with gene therapy/gene edited trials

The objective is to launch a Patient Support Program (PSP) to provide logistical and financial support to patients seeking to enroll in clinical trials. The overall aim is to improve access and retention in clinical trials with an emphasis on underserved populations

Patient Support Program is one component of the 5-year Strategic Plan to create a roadmap for Access and Affordability