

Real Life™

CIRM Access and Affordability Working Group
Patient Support Program Update
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CIRM
CALIFORNIA'S STEM CELL AGENCY

Patent Royalties and License Revenues Paid to the State of California

All royalty revenues received through the intellectual property agreements shall be deposited into an interest-bearing account in the General Fund, ...for the purpose of offsetting the costs of providing treatments and cures arising from institute-funded research to California patients who have insufficient means to purchase such treatment or cure, including the reimbursement of patient-qualified costs for research participants.

February 2022 the AAWG directed CIRM to secure access to \$15.6 million in the “Licensing and Revenue Fund” and provide options for developing a “CIRM Patient Assistance Program” consistent with Proposition 14. CIRM responded by:

- Securing access to funds through the FY 2022/23 state budget process
- Presenting options for the CIRM Patient Assistance Program to the AAWG to inform development of a concept plan
- Developing a draft concept plan in response to AAWG recommendations



Discovery & Gap Analysis: Alpha Clinics Survey, Focus Groups, External and Internal Data Evaluation

- Present the AAWG-recommended Concept Plan for a Patient Support Program (PSP) to the ICOC. The proposed plan will provide:
 - (1) Logistical support for patients being evaluated or enrolled in clinical trials, and
 - (2) Financial support for under resourced and underserved populations in CIRM-supported clinical trials, including the CIRM Patient Assistance Fund (PAF)
- Request the ICOC consider the proposed Concept Plan for developing a request for proposal (RFP) for the CIRM Patient Support Program.
- Highlight the Patient Support Program is one component of the 5-year Strategic Plan to create a roadmap for Access and Affordability.

Plan Rationale: Barriers to Achieving Broad and Equitable Access to Regenerative Medicines



CULTURAL AND SOCIAL DETERMINANTS

- Lower enrollment due to social and economic status [2]
- Employment & education status [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]
- Lack of culturally appropriate information



LOGISTICAL

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



FINANCIAL

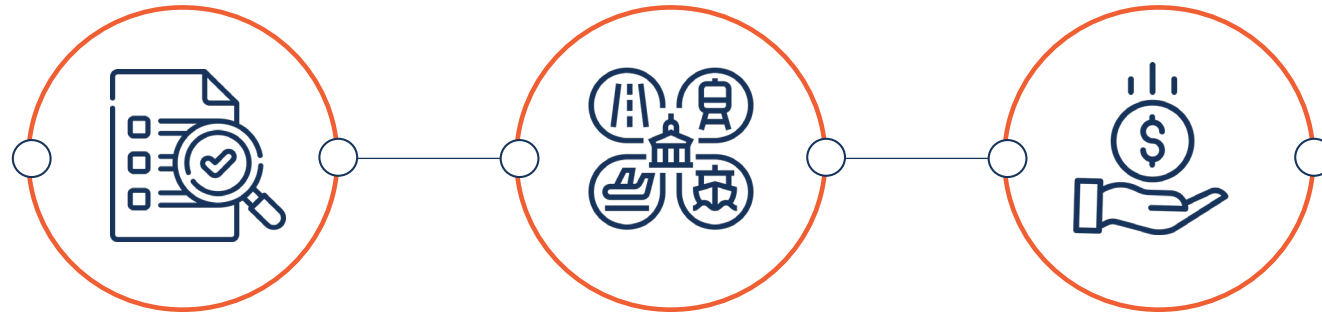
- Cost of regenerative medicines (gene or cell therapies) and insurance benefits may include high copays and lifetime benefit [15]



ABILITY-BASED

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

Three Common Types of Barriers



INFORMATIONAL

LOGISTICAL COORDINATION

FINANCIAL



PSP provides informational (navigation) support to all and determines PAF eligibility

PAF to be deployed within a PSP to provide financial support to under resourced and underserved populations

- Navigating Regenerative Medicine Workshop (2020) / Strategic Planning
- Literature review (barrier identification)
- Key informant interviews including PSP providers
- Internal analysis of CIRM-funded trials
- Survey questionnaire and focus groups
- Engagement with clinical centers through site visits, investigator interviews and IRB discussions
- These efforts are ongoing to inform all Medical Affairs programs

- Large variability in projected patient costs for CGT trials
- Estimated that CGT requires up to 6-9-fold higher expenditures on patient travel and lodging compared to traditional trials
- CGT requires frequent site visits (as many as 100 days for an early phase trial)
- Sponsors evaluate financial needs on a patient-by-patient basis due to extensive differences in financial needs and the nature of the trial
- Burden on trial coordinators may causes disproportionate time focused on reimbursement vs. time spent with patients and trial
- Sites rely on multiple disease funds along with private donations to reimburse for increased patient costs

The CIRM PSP could relieve pressure on current staff and deliver a more systematic and proactive approach to assisting patients.

1 INFORMATIONAL

- A resource for the patient/family working in conjunction with the care team with option to warm transfer to the site. Alleviates undue pressure on site staff and can be viewed as a non-biased conversation about trial options
- Provides standard patient data sets to the sites regarding inquiries

2 LOGISTICAL COORDINATION

- A service to support the needs of patients/caregivers where they would provide coordination of travel, housing, and the reimbursement would allow time to focus on the trial/patients

3 FINANCIAL

- Enrolling and tracking available funding/grants and ensuring patients/families are reimbursed decreases patient/family anxiety and site administrative legwork
- Evaluating the financial needs of patients/families, may reduce the reluctance patients/ families may have about asking for help
- Underserved population financial needs tend to be more significant

AAWG Recommended Scope of Services

- Patient navigation with centrally managed information serving all patients
- Referral or logistical coordination for patients and their families
- Experience across broad range of disease indications
- Capacity to determine PAF eligibility for trial participants
- Data and analytics to track patient interactions and results with real-time reporting capacities
- Cultural adaptation and translation
- Clinical trial experience with academic centers
- Ability to complement Alpha Clinics and Community Care Centers of Excellence
- Adaptive and/or scalable services (e.g. behavioral health)

Who Can Apply?

- For-profit and not-for-profit organizations capable of providing the suite of patient support services defined by the CIRM Patient Assistance Program.
- Applicants must be able to initiate services within 120 days of the final contract.
- Each applicant must have a California location and appropriate state operating license(s).
- All applicants must provide a robust track record of patient support services and activities and be in good regulatory standing.
- Applicants must also have sophisticated data and technology services with multiple back-up capabilities.



Year 1 Discovery

- Finalize Model
- Deploy gap analysis
- Implement basic services
- Create standard metric & monitor outcomes measures



Year 2 Scale 1

- Operationalize services and finances
- Metrics tracking and report to AAWG
- On-going gap analysis



Year 3 Scale 2

- Scale based on additional patient needs
- Metrics tracking and report to AAWG
- Internal Audit



Year 4 Expansion

- On-going operation of suite of patient support services for clinical trials and where applicable, CIRM-funded commercially approved treatments and therapies



Year 5 Assessment

- Continue suite of services
- Provide detailed report to AAWG and ICOC on program impact for California patients

- Service Provider ~ \$300,000 - \$500,000 / year
- Benchmarked to anticipated case volume
- Up to five-year contract with mandated milestones
- Use of Administrative Budget for services (Use of Administrative Funds for Affordability Administrative Staff)
- Funds are included in CIRM Annual Budget

ICOC consider proposed Concept Plan
with a budget (up to \$2.5 million) for
development of a request for proposal
(RFP) for the
CIRM Patient Support Program

Thank you!