Proposition 14 Creates a Patient Assistance Fund

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

### Timeline of Events and AAWG Considerations

- **Feb 2022**: Initiate Budget Process for Patient Assistance Fund
- **May 2022**: AAWG Considers Patient Support Pathways
- **June 2022**: Information Gathering
- **June 2022**: Draft AAWG Patient Support Proposal
- **June 2022**: ICOC Update on AAWG Discussions
- **June 2022**: Governor’s Budget Approved
- **Aug 2022**: AAWG Recommended Concept Plan for ICOC
- **Sept 2022**: ICOC Consideration of PSP Concept
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]
Patient Support Program will Address Three Barriers

Three Common Types of Barriers

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

- **LOGISTICAL COORDINATION**
  - PAF to be deployed within a PSP to provide financial support to under resourced and underserved populations

- **FINANCIAL**
Prior and Ongoing Medical Affairs Research Activates

- 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.
5-year Timeline for Patient Assistance Fund

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
Contract and Budget

• 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!