



CIRM Access and Affordability Working Group
Patient Assistance Program Pathways
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Prop 14 specifically directed that revenues received shall be deposited into an interest-bearing account in the General Fund “Licensing and Revenue Fund”, with those amounts to be spent on “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.”

At the February 9th Affordability and Accessibility Working Group (AAWG) meeting, the group recommended that CIRM request allocation of the existing revenues in the “Licensing and Revenue Fund” to develop a “CIRM Patient Assistance Program.” They recommended:

- CIRM staff to bring proposed options for the CIRM Patient Assistance Program to the AAWG that can be developed into a concept plan.
- That CIRM pursue the best process to access the funds for the 2022/23 fiscal year in order to initiate a program once the AAWG-recommended concept has been approved by the Independent Citizens’ Oversight Committee (ICOC).

- In April, CIRM submitted a Budget Change Proposal (BCP) to the Department of Finance to authorize \$15.6 million California Institute for Regenerative Medicine Licensing Revenues and Royalties Fund for patient assistance program to be spent over 5 years.
- The Department of Finance approved the BCP for inclusion in the Governor's revised budget, which is under review by the legislature this month.
- CIRM has identified three options to be considered by the AAWG and recommended to the ICOC for approval.

- The Patient Assistance Program is one component of the 5-year Strategic Plan to create a roadmap for Access and Affordability.
- The purpose of this presentation is to describe three support program options for patients with financial or hardship barriers to participating in CIRM-supported clinical trials.
- CIRM is requesting the AAWG recommend a lead option for further scoping and development and subsequent presentation for final approval by the ICOC.

Focus on how we get underserved patients enrolled?

Focus on how we help patients stay enrolled?

RECRUITMENT

19% of registered clinical trials are terminated due to failure to reach expected enrollment

RETENTION

80% of trials fail to finish on time despite subject recruitment and retention efforts

85% of clinical trials fail to recruit and retain enough subjects to meet enrollment timelines

**GO/NO
GO**

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

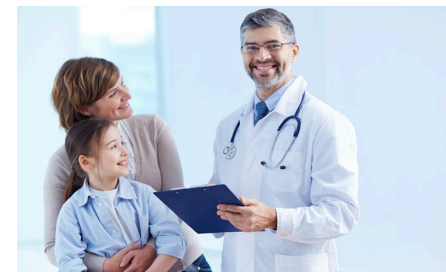
- Participation is limited for elderly,[16] adolescent and young adult,[17] and disabled patients[18,19]

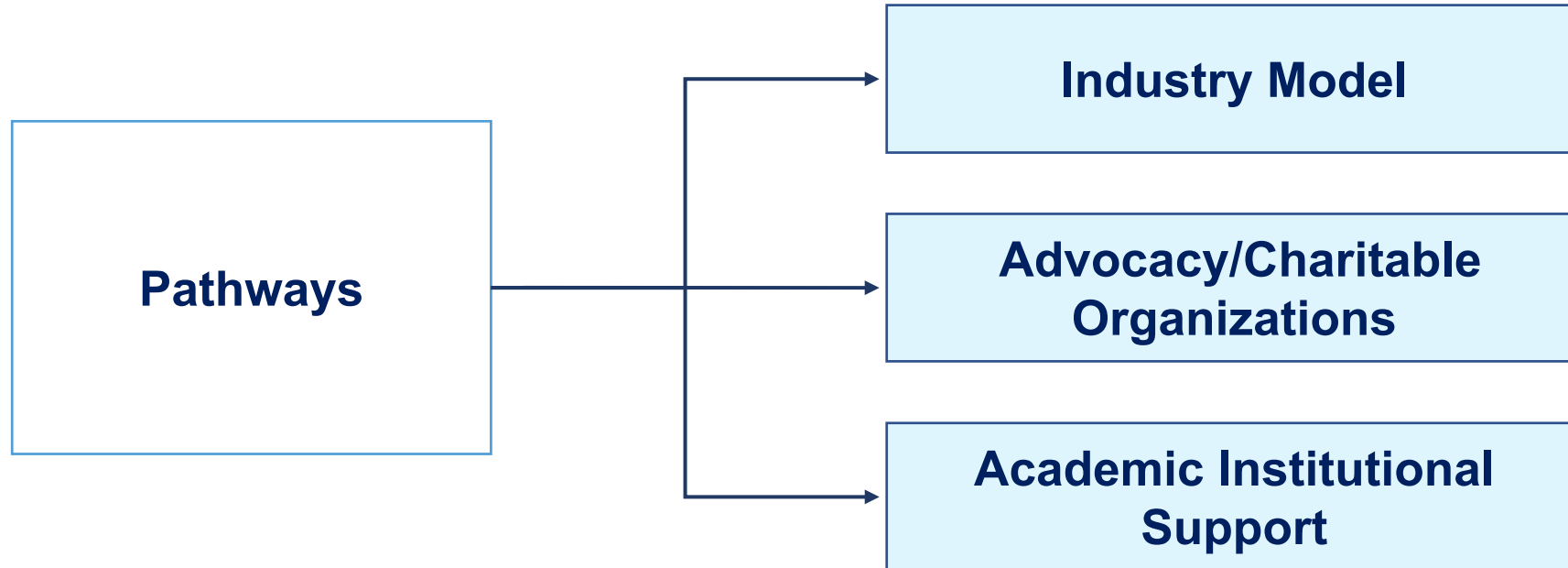


What Items are being Reimbursed or Supported

- Travel expenses
- Accommodation
- Meals
- Childcare
- Out of pocket healthcare expenses
- Ancillary healthcare expenses – e.g. post-administration monitoring
- *Commercial Co-Pay assistance

* Support for transitioning patients to commercially approved products





Established Call Centers with highly trained staff to support patients across clinical and commercial journey:

Clinical Support

Trial triage, information and eligibility, financial support, including transportation, lodging, logistics, post-follow up appointments, ancillary expenses, loss income, childcare, and others.

- Psychosocial support
- Other patient-centric programs
- RWD Collection



STRENGTHS

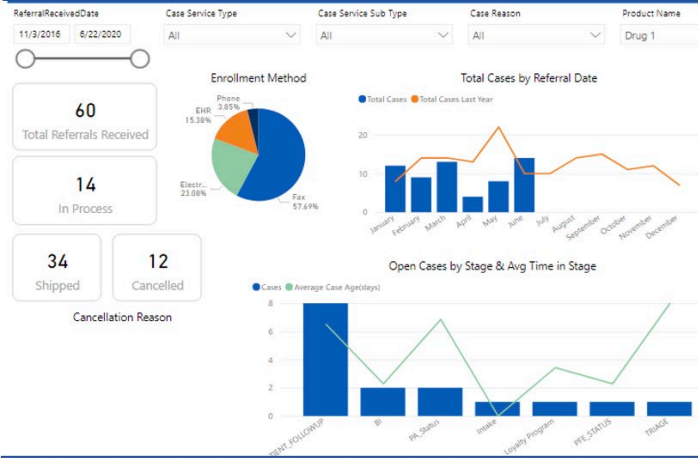
- Case navigator approach
- Scalable/turnkey operations
- State of the art telehealth, software, etc.
- Compliant with state and federal regulations
- Speed of case handling and processing
- Robust analytics to assess performance



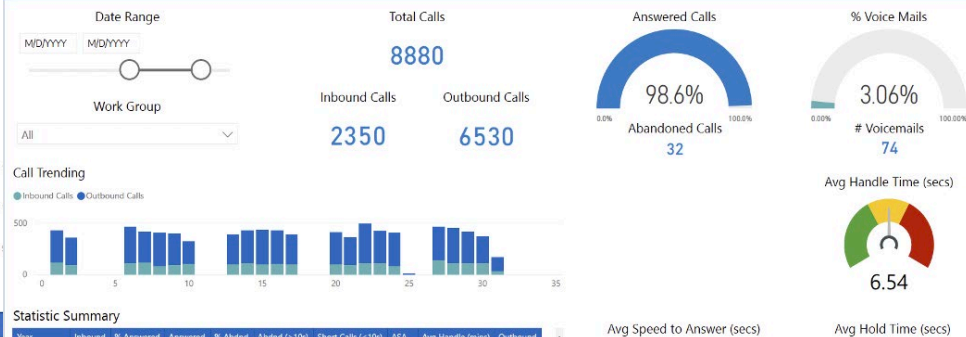
WEAKNESSES

- Ramp up time
- High Demand
- Requires Training
- Expense

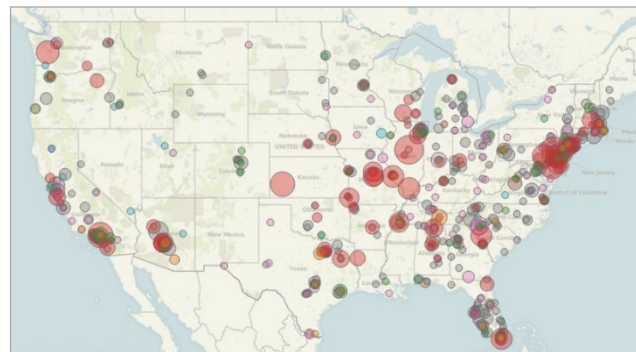
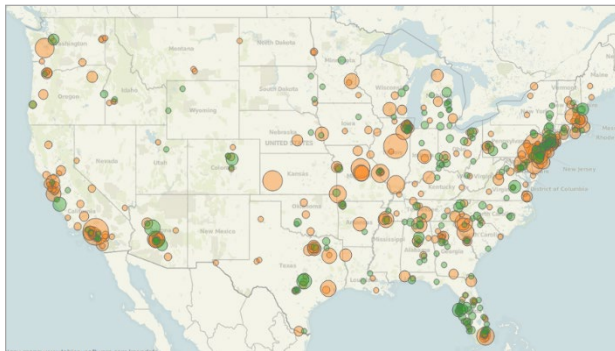
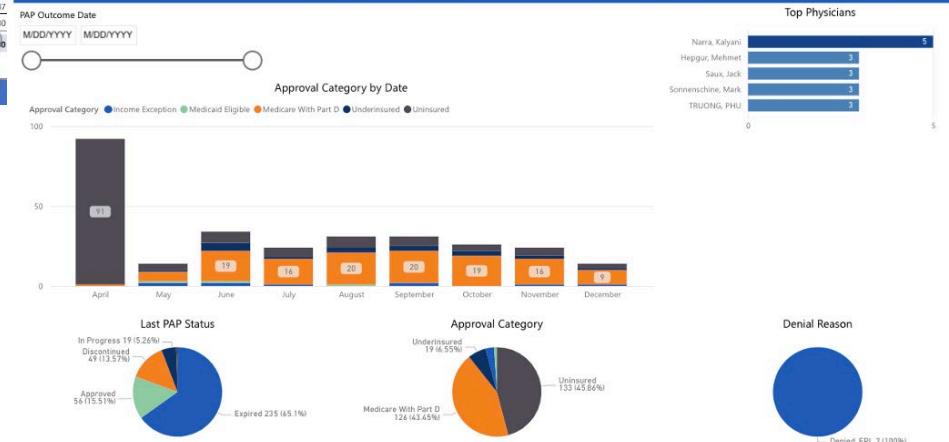
Program Dashboard



Telephony Statistics



Service Outcomes



Numerous Patient Advocacy Groups provide support for disease education, transportation, lodging and logistics associated with clinical trial treatment in underserved populations:

- The Leukemia Lymphoma Society
- The Cholangiocarcinoma Foundation
- NORD, Pancreatic Cancer Network
- The Assistance Fund (TAF)
- Patient Access Network Foundation
- Cancer Care
- Patient Advocate Foundation
- Others



STRENGTHS

- Case Management Approach
- Turnkey operations
- Compliant with state and federal regulations
- Disease-specific funding and education
- Lessons learned and experiences



WEAKNESSES

- Disease specific (most)
- Limited flexibility in design of program
- Limited clinical trial experience
- Limited analytics and scalability

Provide a grant to specific academic clinics to support patients that require assistance with travel, lodging and follow up appointments related to their participation and support in clinical trials for underserved populations

- Examples include academic institutions, Collaborative Groups, etc.



STRENGTHS

- Experience with clinical trial patients
- Patient relationships currently in place



WEAKNESSES

- Limited scalability
- Limited operational experience with administering grants and reimbursement to patients
- Contract with 3rd party providers



Year 1 Discovery

- Determine 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

Thank you and Discussion