Consideration of Concept Plan for Patient Support Program

BACKGROUND

The mission of the California Institute for Regenerative Medicine (CIRM) is to accelerate world class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and the world. The regenerative therapies supported by CIRM are designed to address unmet medical needs that may be curative with a single or limited treatment course in some cases.

Clinical trials for cell and gene therapies are becoming more demanding for patients throughout the entire clinical trial process, from screening and enrollment to long-term follow-up. In particular, increasing financial expenditures by enrolled study participants require added support beyond routine costs for patients and their families. As a result, service providers (for-profit and non-profit) have emerged to provide a range of services to support patients in meeting the new demands of cell and gene therapy trials.

Originating in traditional call centers, Patient Support Programs (PSPs) have grown into highly successful programs that place greater emphasis on providing services to patients throughout the course of their treatment plan. Overwhelming evidence indicates a positive impact of PSPs on clinical trial accrual rates, study participation by minority groups, trial adherence, humanistic outcomes, reduction in healthcare utilization costs, and quality of life. Best-in-class PSPs also provide services related to the needs of the whole patient and may include support with financial stressors and psychosocial, practical, and emotional support. FDA-approved medicines will not be commercially launched in the U.S. without a PSP.

In addition to providing direct patient support, these programs increase the likelihood of trial enrollment, completion of CIRM-supported clinical trials, and protecting the California investment in this research. Due to the high demands of clinical trials, dropout rates can be as high as 30%, which may be attributed to financial costs, long-distance traveling, family commitments, and lack of incentives for study completion. Currently, 85% of clinical trials fail to retain sufficient patients, 37% are terminated before testing even begins due to under-enrollment, 11% fail to recruit a single patient, and 30% of patients who enroll will drop out before the trial is complete. Today, patients enrolled in gene, and cell-edited therapies require support that is specifically customized to their treatment.

Proposition 71, the California Stem Cell Research and Cures Initiative (November 2004), enacted the California Stem Cell Research and Cures Act. This established CIRM for the purpose of making grants and loans for stem cell research and research facilities, and appropriated $3 billion in general obligation bonds to be governed by the Independent Citizens Oversight Committee (ICOC), a distinguished body appointed by California constitutional officers and public university chancellors.

Proposition 14, the California Stem Cell Research, Treatments, and Cures Initiative (November 2020), authorized an additional $5.5 billion in new bonds to continue CIRM funding of stem cell and other medical research and training, stem cell therapy, and delivery of treatments to patients, research facility construction and administrative expenses.

Under these initiatives, CIRM grantees have revenue-sharing requirements for funds they receive from licensing or self-commercializing inventions or technologies that arise from CIRM-funded research. Prop 14 requires that these revenues be deposited into an interest-bearing account in the General Fund 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.” Currently, the CIRM Licensing Revenues and Royalties Fund has a balance of $15.6 million derived from royalty payments.

PURPOSE AND OBJECTIVE

The purpose of this program is to operationalize a Patient Support Program, including the deployment of the Patient Assistance Fund.

The overall objective of this funding opportunity is to establish a statewide Patient Support Program ~~(PSP)~~ with financial and logistical support to all patients being evaluated or enrolled in CIRM-supported clinical trials to improve access, enrollment, and retention in clinical trials with an emphasis on underserved populations. These services include but may not be limited to:

- Clinical trial navigation, directing patients to appropriate CIRM-supported clinical trials based on trial criteria/eligibility for appropriate determination/potential selection.

- Evaluating patient/family needs for financial support for non-covered services or products associated with CIRM-supported trials and directing reimbursements based on Federal Poverty Level (FPL) criteria. ~~FPL will be assessed utilizing a third-party screening tool (e.g., Experian or TransUnion) to validate patient claims of income and household size.~~

- Assessing patient/family needs regarding logistical/travel support and administering reimbursements for CIRM-enrolled patients for attending

required medical appointments. This support includes transportation/travel expenses, such as gasoline, tolls, parking, airfare, taxi, train, lodging, and meals during travel.

Phase 1 of the award will collect intelligence on the status of patient support programs, launch basic services to meet the needs of regenerative clinical trial patients, and identify additional potential patient services for AAWG consideration.

Phase 2 will scale additional patient support services, which may include ongoing nurse navigator support for the psychosocial, emotional, and practical needs of patients and their families~~.~~

AWARD INFORMATION

How Is the Program Structured?

The first phase of the program will award an organization having suitable expertise with a grant to provide patient support services. These will address the financial and logistical bottlenecks often experienced by patients and their family members enrolling in or participating in CIRM-funded cell and gene therapy trials. The awardee is expected to have demonstrated expertise in the services of standard patient support programs ~~using a HUB service model~~:

- Maintaining a call and support center:
  - Provide live support to handle inbound inquiries during normal program hours. The call center shall operate Monday through Friday from 8:00am to 5:00pm Pacific Time, exclusive of recognized holidays.
  - Provide individualized customer service to callers pursuant to the business rules documents (“BRD”), standard operating procedures (“SOPs”) and specific operational process flows (“PFs”).
  - The service provider ~~HUB vendor~~ will obtain patient and/or health care provider authorization and consent for participation in the program using CIRM-approved language that is compliant with HIPAA and all other applicable laws and regulations.
- Assessment of eligibility for financial assistance:
  - The service provider ~~HUB vendor~~ will utilize BRD to determine approval or denial and provide resulting reimbursement *via* debit card for expenses related to non-covered services or products as a result of the associated CIRM-support trial.
  - The service provider ~~HUB vendor~~ will utilize BRD to determine approval or denial and provide resulting reimbursement *via* debit card for expenses for logistical/travel support based on clinical trial appointments, testing, and related requirements.

- The service provider ~~HUB vendor~~ shall provide CIRM with access to standard and customized reports. Access to the reports should be available 24/7 from anywhere that internet access is available to the user.

After granting the award, Phase 1 of the program will entail preparing BRD, SOPs, PFs, compiling educational materials, creating standard metric reports, and staff recruitment and training. When the operational system, BRD, SOPs, PFs are in place, system testing will be implemented to assess operational readiness.

Phase 1 services will be activated through post-testing and staff training. The program will be communicated and advertised through Alpha Clinics and the CIRM website. Real-time monitoring will occur at the time of activation to identify any gaps or opportunities for additional services.

Phase 2 of the program will include further expansion of patient services and initiation of additional patient financial services based on real-time metrics reporting provided by the service provider ~~HUB vendor~~, ongoing internal gap analysis, and patient experience surveys. Call center metric reporting and real-time gap analysis results for trial participants will be provided to the Accessibility and Affordability Working Group (AAWG) members.

How will Funds be Awarded~~is Funding Provided~~?

Awards will be made in the form of a grant. Funds will be disbursed pursuant to a CIRM Notice of Award. The first payment will be issued upon initiation of an award and subsequent payments will be disbursed on a regular interval at CIRM's option. Continued funding is contingent upon timely progress, as outlined in the project milestones and timeline established under the Notice of Award.

The PSP service provider will be chosen through a competitive RFA~~P~~ process and awarded to a for-profit or not-for-profit organization that can provide patient support services and other requirements as outlined in this concept document ~~according to the concept plan proposed by the AAWG members and approved by the CIRM Board~~.

During a 5-year period, the program is expected to support hundreds of patients in need as they participate in the growing number of CIRM-supported clinical trials.

How will the Program be Monitored?

Metrics used to evaluate the program will include industry-standard monthly reports on contact volume and type, speed to access to clinical trials, enrollment metrics, type and frequency of reimbursement requests, the average time to reimbursement, and average time to case fulfillment. These metrics will be used to evaluate the impact of the program and assist the patient enrollment processes of CIRM-supported clinical trials:

- 3rd Party vendor/call center telephony metrics: speed to answer, hold time, and service turnaround time

- Case insights
- Financial assistance services demographics and utilization
  - Non-covered services
  - Logistics/travel
- Alpha Clinic referral
- Benefit investigations
- Program dashboard
- Quality metrics of case managers
- Clinical trial retention scores
- Patient satisfaction surveys

Throughout the 5-year period, CIRM will monitor pre-defined metrics and deliverables that indicate the impact of this Fund on patient access to clinical trials and/or treatments arising from CIRM programs. These outcomes will be reported to the CIRM AAWG and ICOC.

Program Requirements

All applications for this funding opportunity will be required to provide statements describing:

1. **General Information**
   a. The organizational structure for supporting this program, the applicant's~~ir your~~ experience with providing exceptional management to organizations for specific programs, and the average size and scope of the applicant's~~your~~ typical/average patient support ~~HUB~~ program serviced by the applicant~~that you are currently servicing~~.
   b. If the applicant~~they you~~ are currently provid~~esinge~~ services in the cell and gene therapy space ~~to any company that might be determined to be a direct competitor~~.
   c. If the applicant~~you~~ had~~ve~~ any such arrangements, provide details if the contracts were terminated by either party.
   d. State ~~your~~the applicant's lead time requirements for the operationalization of the service provider ~~a HUB~~ as outlined.
2. **Capabilities and Experience**
   a. Hours of operation, after-hours protocol, time zone management.
   b. Team structure includes the number of call center agents (reimbursement counselors, patient care coordinators, case managers, etc.) and the number of call center supervisors and/or managers in each service location.

c. The percentage of staff that works from home *vs*. working out of an office. Please describe this structure and, if applicable, how ~~you~~the applicant determines who works from home.

d. ~~Explain your~~The applicant's HIPAA compliance management process.

e. The applicant's~~Indicate your~~ years of experience with clinical trial patients, rare diseases, and the types of programs geared towards rare disease patients, including overall program size and disease areas.

f. Process and turnaround times for verifying insurance benefits.

g. A description of the applicant's~~Please describe all~~ quality measures (efficiency and effectiveness) utilized for program operations.

h. A description of the applicant's ~~Please describe your~~ reimbursement/stipend payment process for patients that are approved for funding.

i. A description of ~~Describe~~ current in-house design functionality or partnerships with other third-party vendors to administer aspects of proposed ~~your~~ services.

j. List ~~the number~~ of programs for which the applicant~~you~~ provides travel, lodging, and transportation.

k. The applicant's ~~What is your~~ process for uninsured patient screening, and how the applicant ~~do you~~ supports patients who qualify for insurance but have not yet applied.

l. A description of ~~Describe~~ the non-English language capabilities of patient-facing staff by language and role.

m. A description of ~~Describe~~ technology tools utilized and any innovations in development to improve access solutions, retention in clinical trials, and how they are delivered.

n. A description of the applicant's~~Describe your~~ technology or processes that will eliminate duplicative costs with other CIRM-funded programs.

3. Data and Technology

a. A l~~L~~isting of all systems used in the collection, aggregation, and delivery of required data sets (internal or commercial systems) with system scalability to meet product growth over the program lifecycle.

b. A description of ~~Describe~~ maintenance of historical data, including audit trails.

c. A description of~~Indicate~~ the de-identification process and compliance with HIPPA/HITECH legislation.

d. A description of ~~Describe~~ the process for quality control and how the applicant~~you~~ ensures that data is delivered timely with >99% completeness and accuracy. A description of ~~Describe your~~ the applicant's current performance metric for quality and timeliness.

e. A description of the applicant's~~Describe your~~ data security measures and certifications in place today. ~~List all security certifications.~~

f. A description of ~~Please describe~~ the applicant's ~~your~~ standard reporting package and ~~provide~~ an example of the types of reports that are considered customized.

g. A description of ~~Please describe your~~ of the applicant's phone technology and specifically what unique capabilities are~~you~~ offered.

What Activities will CIRM Fund?

CIRM funds will support the following activities under this opportunity:

- Clinical trial navigation to support ~~steering~~guiding patients to appropriate clinical trials based on trial criteria/eligibility for appropriate determination/potential selection. Clinical trial navigation will include partnerships with Alpha Stem Cell clinics and other community organizations.
- Assessing patient/family needs regarding financial support and administering supplemental reimbursements based on federal poverty level (FPL) criteria. Some of the reimbursements will include:
  - travel expenses
  - lodging
  - childcare
  - meals
  - additional medical expenses
  - other allowable reimbursable costs

~~• Travel expenses to and from the trial:~~

~~○ gasoline~~
~~○ parking~~
~~○ tolls~~
~~○ taxi~~
~~○ car service (Uber/Lyft)~~
~~○ airfare~~
~~○ subway card~~
~~○ train~~

~~• Accommodations during trial~~

~~○ lodging (hotel, Airbnb™)~~

- ~~Childcare~~
  - ~~babysitter~~
  - ~~additional daycare~~

The Award Amount and Duration

CIRM will issue only one award to an organization that will fund direct project costs of up to $2,500,000 for a duration of up to five years. Direct project costs must be adequately justified and are subject to adjustment prior to the issuance of an award based on assessments by the Accessibility and Affordability Working group (AAWG), the CIRM team, or by CIRM's Governing Board. The award will not provide direct facilities costs or indirect costs.

CIRM will fund a PSP service provider for up to 5 years.

Each year, the program will be audited to assess the overall impact of the program and its level of performance. Real-time data will be used to identify additional patient services and brought to the attention of the AAWG for consideration.

ELIGIBILITY

Who can Apply

For-profit and not-for-profit organizations that have a full suite of patient support services to meet the defined needs of the CIRMs Patient Assistance Program.

Applicants must be able to initiate services within 120 days of final contracting.

Each applicant must have an appropriate California operating license~~.~~ and a California operating location: Applicants must conduct a majority of the PSP's operations from a facility permanently located within California that is adequately equipped to provide the required services.

All applicants must have demonstrated a robust track record of providing patient support services and activities and be in good regulatory standing.

Applicants must have robust data and technology services with multiple backup capabilities.

For-profit organizations must demonstrate solvency. For-profit organizations must provide documentation that shows 180 days cash on hand from date of application submission. The determination of solvency will be made at CIRM's sole discretion

Application must be accurate and complete. All required components of the application must be completed and may not contain false or inaccurate information.

Diversity Equity and Inclusion (DEI)

Applicants must submit a robust DEI plan

Applicants must provide a clear and robust plan for program engagement, helping sites with retention into the clinical trial process and implementing assistance programs to address barriers to program participation faced by underserved populations.

Please explain ~~your~~ applicant's experience addressing DEI and how the applicant~~you~~ will operationalize its~~your~~ plan~~s~~. ~~Plans~~ A DEI plan shall include, for example:

- Alliances with community clinics
- Designating community liaisons
- Social network engagement
- Distribution of culturally sensitive materials in multiple languages
- Patient navigators
- Other state-run programs for underserved patient populations.

What are the operational components of applicant's~~your~~ DEI program, and how do~~es~~ the applicant~~you~~ measure its impact?
How will the applicant~~you~~ develop culturally sensitive responsive materials?
How does applicant's~~your~~ DEI plan add value to the ~~RFP~~program?

Applicants must demonstrate activities for building cultural sensitivity on the team and/or at partner institutions.
Activities should include:

- Implementing published guidance for cultural sensitivity in patient care
- Training for team members in culturally responsive clinical skills
- Convening a panel for guidance and oversight

How will the applicant facilitate engagement, participation, and retention of underserved patient populations in the Patient Support Program?

If the applicant~~you~~ does not~~don't~~ have a DEI track record~~plan~~, how will~~do~~ the applicant~~you~~ ~~plan to~~ mitigate this lack of experience? How will applicant ~~you~~ operationalize the program, given the DEI requirements? What is the applicant's~~your~~ mitigation plan with details on ~~how you will~~ identify~~ing~~ risks that need attention?

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