

CURE

Beyond
CIRM 2.0
CALIFORNIA'S STEM CELL AGENCY

now it's personal



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Vice President Portfolio Development and Review
Agenda Item #6
Alpha Stem Cell Clinics Presentation

HOPE



Challenges to Accelerating Stem Cell Therapies

Challenge: Moving stem cell treatments through IND-enabling studies to clinical trials takes disproportionately more time when compared to non-cellular treatments.

Solution: As integral components of CIRM's Strategic Plan, CIRM's infrastructure programs are designed to overcome the obstacles that currently slow the progress of translating stem cell research to high quality clinical trials.



CIRM Infrastructure Programs

	Program	Goal	Role
Stem Cell Center	Translating Center	Shorten time to clinical testing	<ul style="list-style-type: none">• Process Development• IND-enabling activities
	Accelerating Center	Accelerate clinical research	<ul style="list-style-type: none">• Regulatory Submissions• Trial management
	Alpha Clinics Network	Conduct high quality clinical trials	<ul style="list-style-type: none">• Specialize in cell therapy clinical trials• Develop AVARs (Accelerating and Value Add Resources)



Alpha Stem Cell Clinics Network

- Network launched in December 2014 and active by Q1 2015
- Established at three sites
 - City of Hope
 - UCSD
 - UCLA/UCI Consortium



Key Functions of an Alpha Clinic

- Contracting / clinical trial agreements
- IRB submissions and approval
- Patient recruitment, screening, enrollment
- Clinical trial monitoring support
- Patient coordination and scheduling
- Site data management

Services are proportional to the needs of the projects



Network Expansion

- Add up to two sites to the network
- Include new physician training component
- Enhance value to the network
- Create sustainable program
- Provide up to \$8 million over four years

Alpha Stem Cell Clinics Network Expansion

REVIEW PROCESS

Protocol for Review

- Initial discussion – 20-25 minutes
- Applicant Presentation “Pitch” – 5 minutes
- Q&A with Applicant – 25 minutes
- Wrap-up discussion and scoring – 15 minutes
 - Repeat for each application

Review Criteria

- Will the proposed Alpha Clinic accelerate completion of stem cell therapy clinical trials, enhance the value of the network, and be sustainable?
- Has the applicant developed a plan designed to successfully establish and operationalize the Alpha Clinic?
- Is the proposal feasible?

Scoring System for 2.0 Applications



- **Score of “1”**

Exceptional merit and warrants funding.

- **Score of “2”**

Needs improvement and does not warrant funding at this time but may be resubmitted if the CIRM board has not approved an application.

- **Score of “3”**

Sufficiently flawed that it does not warrant funding.

Applications are scored by all scientific members of the GWG with no conflict.

Guidance to GWG Reviewers



- CIRM is ideally looking to expand the program with 2 new sites.
- You are not required to select a winning application. If none are deserving of a “1”, do not score any in that tier.
- Based on your recommendations, CIRM’s Governing Board will determine which applicants will receive an award or be given the opportunity to resubmit a revised application.

GWG Vote on Review Process

1. *All members:* “The review was scientifically rigorous, there was sufficient time for all viewpoints to be heard, and the scores reflect the recommendation of the GWG.”
2. *Patient advocate members:* “The review was carried out in a fair manner and was free from undue bias.”

Vote was unanimously in favor on both statements for all applications reviewed in this cycle.

INFR4-10313



Title: The Alpha Clinic: A Partner in the Advancement of Cell Therapy Research

GWG Score: 1 Exceptional merit and warrants funding

- Votes for score of 1: **7**
- Votes for score of 2: **4**
- Votes for score of 3: **2**

Funds Requested: \$7,999,999

INFR4-10314



Title: Alpha Stem Cell Clinic for Northern and Central California

GWG Score: 1 Exceptional merit and warrants funding

- Votes for score of 1: **13**
- Votes for score of 2: **0**
- Votes for score of 3: **0**

Funds Requested: \$7,905,671

INFR4-10361



Title: CIRM Alpha Stem Cell Clinic

GWG Score: 1 Exceptional merit and warrants funding

- Votes for score of 1: **9**
- Votes for score of 2: **6**
- Votes for score of 3: **0**

Funds Requested: \$7,999,999