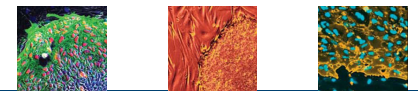


CIRM Alpha Stem Cell Clinics Network- Alpha Clinic Award RFA 13-06

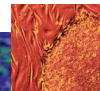
**October 23, 2014 ICOC Meeting
Agenda Item #8
Summary and GWG Recommendations**

GWG Review Date: September 16-17, 2014



The Four Part Test for all CIRM initiatives

- Will it accelerate the development of a stem cell treatment?
- Will it increase the likelihood of developing a successful treatment for patients?
- Will it fill an unmet medical need?
- Is it efficient?



Guidelines Given to the GWG for Funding Recommendations

- ICOC approved up to \$55M to fund up to 5 Alpha Clinics.
- There is no predetermined number of applications that should be recommended for funding.



Goal of the Alpha Stem Cell Clinics Network

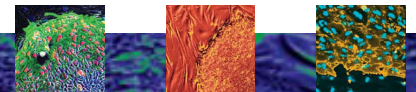


The network is composed of Alpha Clinics and a Coordinating and Information Management Center (CIMC).

This review is focused on the **Alpha Clinic Award** (*the ICOC rescinded its approval of the CIMC Concept Plan and approved a more focused plan; a new RFA for that is in development*)

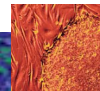
Value Add by:

- **Accelerating** the development and delivery of stem cell therapies
- **Leveraging** existing assets to form an **efficient, scalable** and **sustainable** Alpha Stem Cell Clinic well positioned to attract and conduct **high quality stem cell trials**



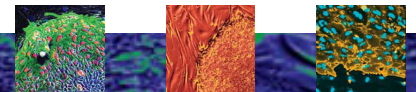
Eligibility

- California Institution
- Program Director- active medical license and hospital privileges
- At least two Lead Clinical Trials
 - ✓ IND submitted for at least one at time of application
 - ✓ Committed source of funding and clinical trial agreement or sponsor letter of commitment
 - ✓ Trial initiation within the first year
 - ✓ Stem-, progenitor- or gene modified cell product



Guidelines for Review

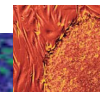
- Based on the initial preliminary review of the full applications that were submitted in March, the following questions were asked of the applicants in July:
 - How will this award accelerate/improve your lead clinical trials?
 - What would be the benefit to subsequent clinical trials conducted at your site?
 - How will your ability to recruit new trials be improved?
 - What metrics will be used to measure success?
- Reviewers were instructed to take these responses into account when evaluating the proposals



Scope of Activities for Alpha Clinic Sites

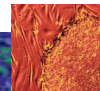
Site Operational Support for Clinical Trials

- Patient recruitment, screening, enrollment
- Clinical trial monitoring support
- Patient coordination and scheduling
- Site Data Management
- Work with other Alpha Clinic Sites
- Support the conduct of the lead clinical trials and pipeline of clinical trials



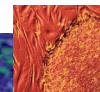
Out of Scope Activities

- Construction of new or renovation of existing facilities
- Preclinical research and IND-enabling studies
- Bioprocessing development
- Cell manufacturing
- Clinical trial costs, e.g. patient costs, hospitalization, pharmacy, labs, procedures



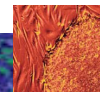
Review Criteria

- **Responsiveness to the RFA**
Is the proposal, backed by the responses to the supplemental questions, responsive to the goals and objectives of this RFA?
- **Institutional Support and Sustainability Plan**
In-kind or matching contributions, leverage existing resources, support for the Program Director's role and commitment to maintaining the clinic beyond the 5 years of CIRM funding.
- **Alpha Stem Cell Clinic Team**
Institutional and Team track record in efficient and high quality clinical trial execution of FDA regulated investigational products.



Review Criteria

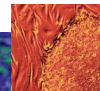
- Implementation and Operational Plan
 - Feasibility of plan and timeline for set up of the clinic and conduct of Lead Clinical trials.
 - Are the activities appropriate and adequate to support the proposed trials?
 - Is the proposed budget appropriate and competitive for these planned activities?
 - Feasibility and strength of plans to scale capacity and to support a pipeline of trials in a broad range of disease targets and indications.



Review Criteria

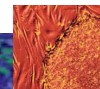
Alpha Stem Cell Clinic Lead Clinical Trials

- Quality and relevance to CIRM's mission
- Address important unmet medical need(s)
- Likelihood of successful trial conduct given the institution and team
- Feasibility of an active trial within the first year



Overview of Applications:

- 7 Applications, each proposing a minimum of 2 Lead Clinical Trials
- The Lead Clinical Trials will represent the “starting point” and future “pipeline” trials will be conducted by the Alpha Clinic.
- 18 Lead Clinical trials in the following Disease Areas:
 - Neurologic Injury (Spinal Cord Injury, Traumatic Brain Injury)
 - Neurodegenerative Disease (Huntington’s Dis., ALS)
 - Diabetes
 - Skin Disease
 - Cardiac and Peripheral Vascular Disease
 - Pulmonary Disease
 - Hematologic Disease
 - Cancer
 - AIDS



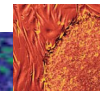
Overview of the GWG Meeting

- Focus of the review and discussions was whether the given project would accelerate stem cell therapeutic development and whether the proposed plans and resources would add value to what is already available
- GWG discussed “acceleration” and “value” with respect to the lead trials and/or acceleration and value to future pipeline trials
- GWG recommended additional budget review to delineate “new resources” that were being added versus those already covered by a trial Sponsor or existing institutional or other network resources



The GWG Motion

“To forward to the ICOC the Grants Working Group funding recommendations for ‘CIRM Alpha Stem Cell Clinic Awards’ as set forth in the displayed table, with the recommendation that the respective proposed budgets be reviewed to eliminate costs associated with the CIMC, and eliminate costs covered by trial sponsors, e.g., clinical operations (excluding patient recruiting costs).”



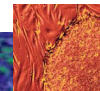
CIRM Recommends

To be responsive to the GWG motion of:

- removing CIMC costs from the budget, AND
- preventing duplicative clinical trial spending (covering trial costs already being paid for by a sponsor),

CIRM recommends funding Tier 1 applications:

- with a requirement to recast the budgets, AND
- with a funding cap of \$8 million per award.



GWG recommendations

TIER 1 BUDGET \$33,578,323

TIER 2 BUDGET \$11,000,000

Application #	Title	SCORE	Median	SD	Range		Budget	Tier
					Low	High		
AC1-07659	The Innovation-Alpha Clinic for Cellular Therapies (I-ACT) – A Program for the Development and Delivery of Innovative Cell-based Treatments and Cures for Life-threatening Diseases.	85	85	8	70	100	\$10,975,088	1
AC1-07764	Alpha Stem Cell Clinic for the Development of Regenerative Therapies	77	76	7	65	85	\$11,673,826	1
AC1-07675	Alpha Stem Cell Clinic (ASCC) Consortium	75	75	9	50	85	\$10,929,409	1
AC1-07650	The Regenerative Medicine Alpha Stem Cell Clinic Program Proposal	68	64	8	60	85	\$11,000,000	2
AC1-07788	Stem cell clinic for the treatment of genetic and chronic diseases, and injury	*					\$10,904,643	3
AC1-07637	Alpha Clinic - a Hub for Stem Cell Therapies	*					\$10,779,909	3
AC1-07742	CIIRM Alpha Clinic	*					\$10,963,076	3

