CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
THE STATE STEM CELL AGENCY

MEETING NOTICE and AGENDA REGULAR MEETING of the SCIENTIFIC AND MEDICAL ACCOUNTABILITY STANDARDS WORKING GROUP OF THE INDEPENDENT CITIZENS OVERSIGHT COMMITTEE TO THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE Organized Pursuant to the CALIFORNIA STEM CELL RESEARCH AND CURES ACT

California State Stem Cell Agency
Standards Working Group Meeting

Tuesday October 1st, 2013
9:00am to 12:00pm

Main Location:
CIRM
210 King Street - 3rd Floor
San Francisco, CA

1. Welcome

2. Call to Order & Roll Call

3. Review Concept Proposal for Alpha Clinics Request for Applications and consider regulatory amendments emanating from 2013 ESCRO Workshop(PDF).

4. Review DISCUSS Project draft recommendations, receive comments from SWG and consider endorsement of project.

5. Update on progress of CIRM iPSC Bank and donor consent protocol.

6. The Committee will accept public testimony on any matter under its jurisdiction that is not on the agenda, but the Committee cannot act on any such matter at this meeting.

THE ORDER OF BUSINESS MAY BE CHANGED WITHOUT NOTICE.
NOTICE
The California Institute for Regenerative Medicine and its Independent Citizens Oversight Committee, and any subcommittees thereof, comply with the Americans with Disabilities Act (ADA) by ensuring that the meeting facilities are accessible to persons with disabilities, and providing that this notice and information given to the Members of the Committee is available to the public in appropriate alternative formats when requested. If you need further assistance, including disability-related modifications or accommodations, you may contact Amy Cheung at the California Institute for Regenerative Medicine at 415-396-9100 no later than the day prior to the meeting.

Questions or requests for additional information prior to the Scientific and Medical Research Funding Working Group meeting may be referred to at Amy Cheung acheung@cirm.ca.gov or 415-396-9815.
This meeting agenda is also available on the website for the California Institute for Regenerative Medicine at http://www.cirm.ca.gov.

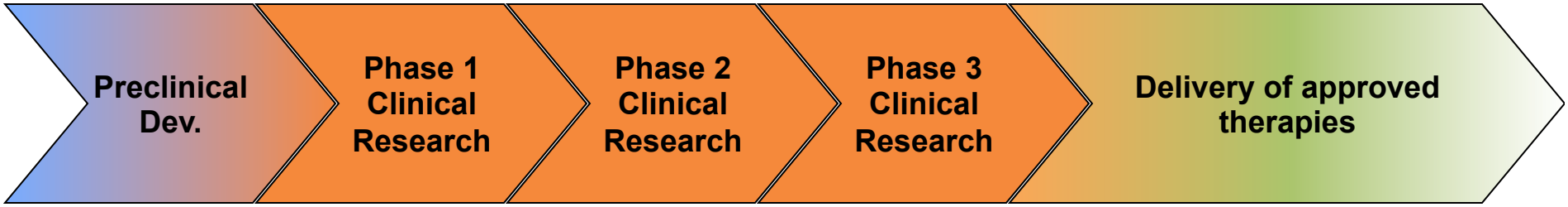
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

The state stem cell agency

Alpha Stem Cell Clinics Network: Standards Working Group Considerations

October 1, 2013

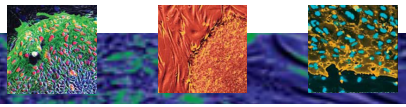
Challenge: unmet needs in clinical infrastructure for stem cell therapies



- **Development of stem cell therapeutics is on the rise**
- **Testing and delivery of stem-cell based products present unique challenges**

Alpha Stem Cell Clinics Network Attributes

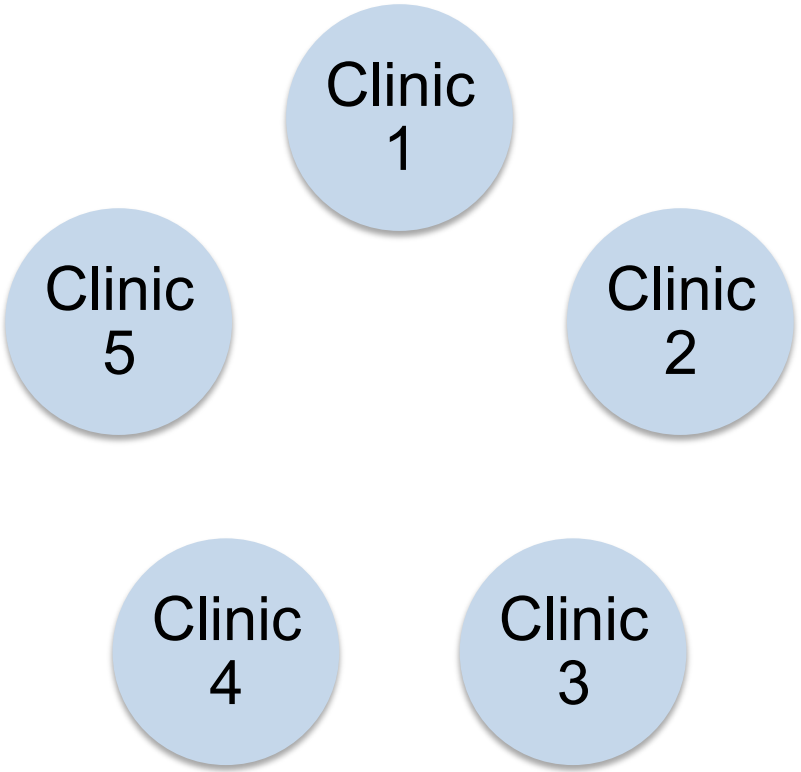
- **CLINICAL TRIALS:** Develop resources designed for effective and efficient design and execution of clinical trials for investigative stem cell products
- **DELIVERY OF THERAPIES:** Become a center of excellence for delivery of stem cell-based therapies that have been proven safe and effective
- **DATA AND INFORMATION:** Compilation of information about clinical trial experience and outcomes, and data to inform research, clinical, regulatory and reimbursement decisions
- **INFORM THE PUBLIC:** Education, outreach and training about clinical trials and available therapies, and potential dangers of unproven procedures
- **HEALTHCARE ECONOMICS:** To serve as a “proving ground” to develop business models, and to develop evidence base and strategy for reimbursement



Focus of Alpha Stem Cell Clinics Network

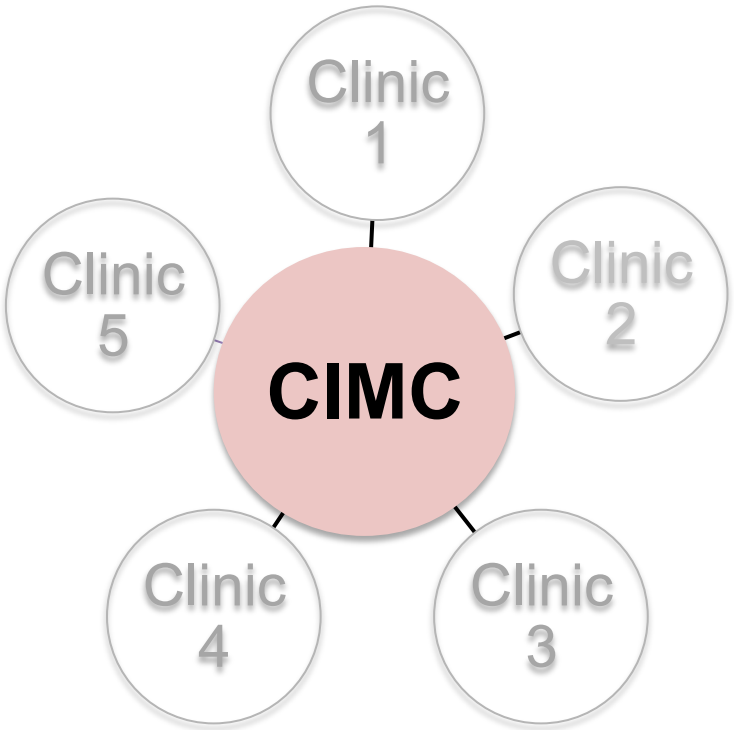
- Stem-cell derived products that are novel as opposed to modifications of therapies in current medical practice
- Procedures that require transplantation or infusion of cells, as opposed to small molecules or biologics

Alpha Clinics: five sites distributed throughout California



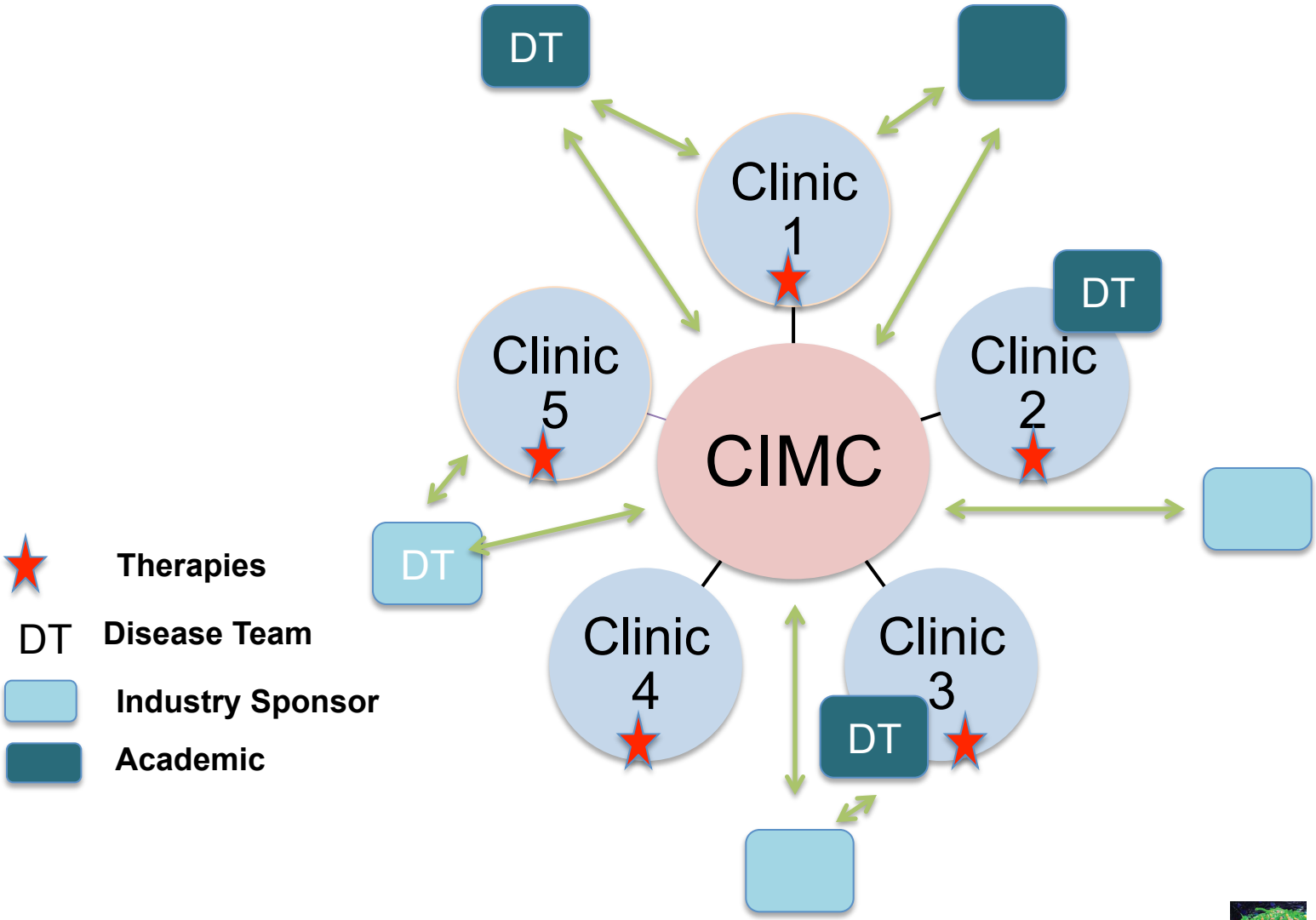
- **Conduct clinical trials**
- **Patient Education & Counseling**
- **Delivery of approved therapies**

Coordinating and Information Management Center (CIMC)

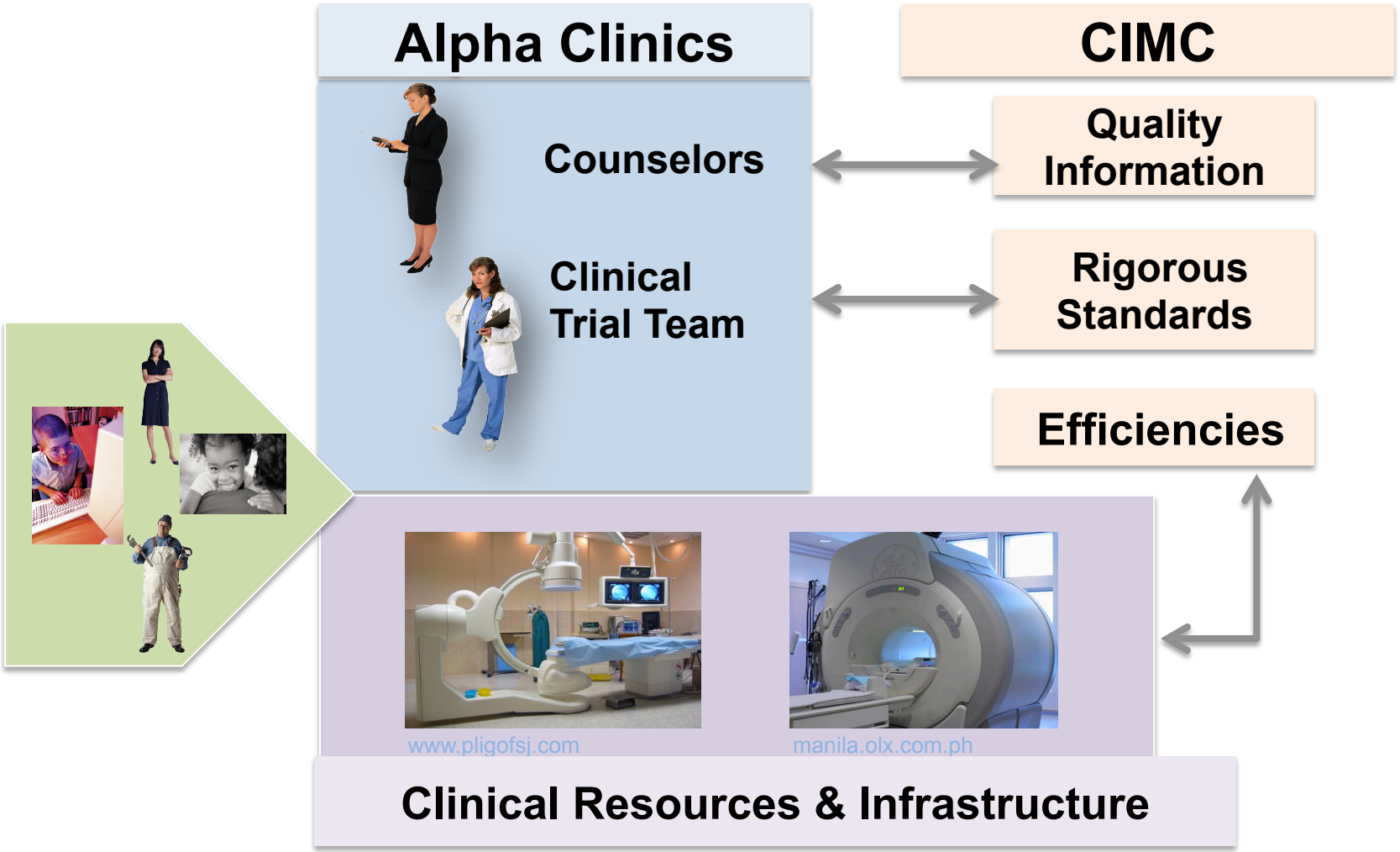


- **Outreach, education and training (OET)**
- **Consulting services**
  - **Clinical**
  - **Regulatory**
  - **Biostatistics**
- **Patient Registry and Database**
- **Healthcare economics and business development**

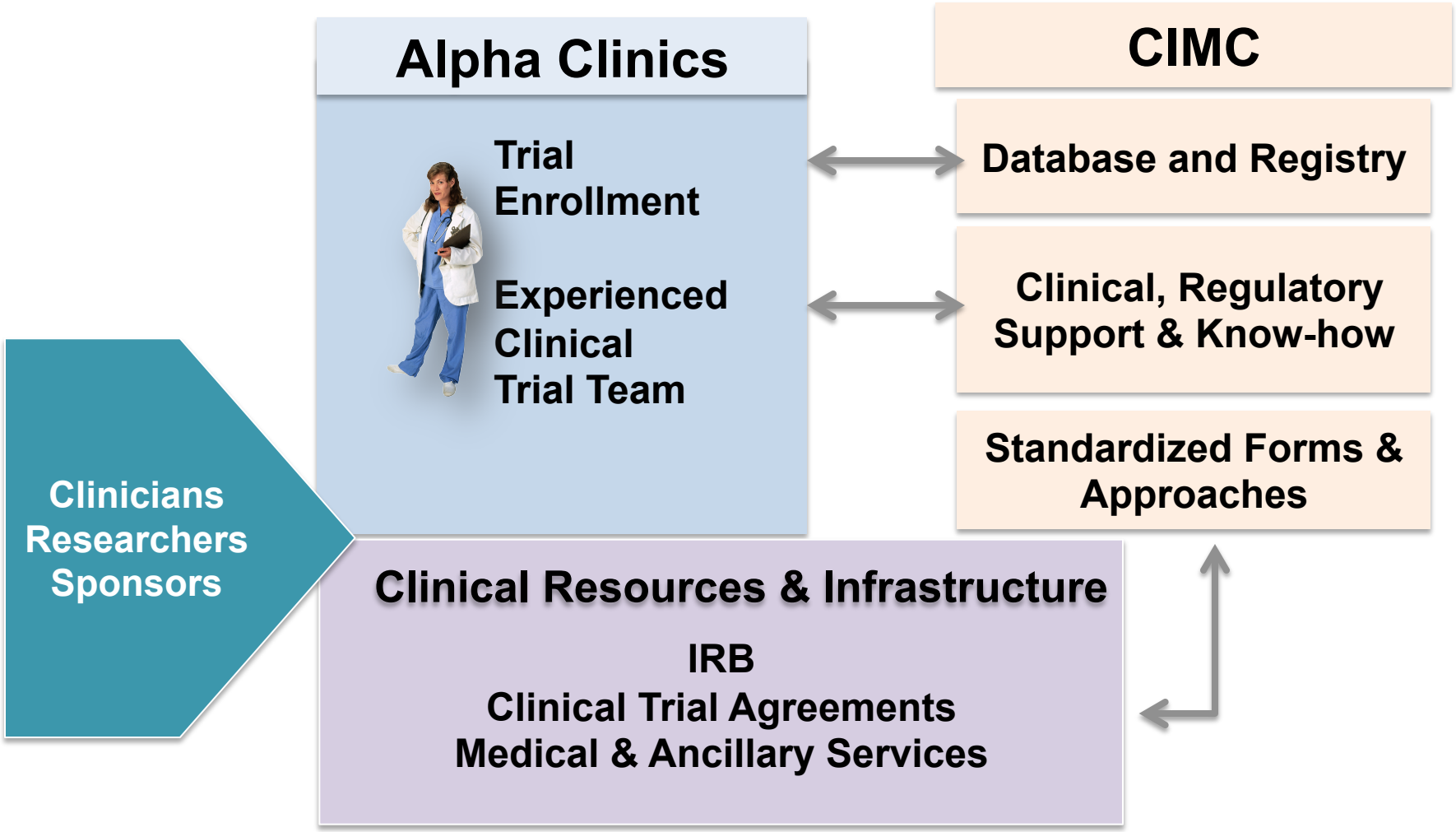
Long-term vision: A robust network to test and deliver stem cell- based therapies



Alpha Clinics Would Serve Patients Seeking Stem Cell Treatments



Network Resources & Efficiencies for Stem Cell Clinical Research



Provisional Time Table for Alpha Clinics Network RFAs

Release of RFAs	October 2013
Letters of Intent due	December 2013
Applications due	March 2014
GWG Review of Applications	May 2014
Funding Recommendations to ICOC	July 2014

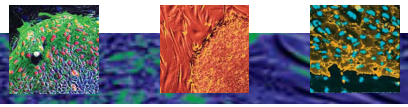
Issues for Consideration by SWG

- Multiple workshops and meetings were convened to consider oversight of cell-based clinical trials. (Alpha Clinic Workshop 11/2012, University of California Biomedical Research Acceleration Integration & Development meetings, ESCRO Workshop 6/2013).

- Participants described efforts to ensure effective review and oversight of clinical trials. These efforts were “IRB-centric” and included expertise in cell-based therapy.

- IRBs **always** have responsibility for the review and oversight of clinical trials.

- Approaching review and oversight in manner consistent with national policy provides efficiencies and effectiveness for Alpha Clinic Cell Clinics Initiative.



Options for Revision to Section 100070: CIRM Medical and Ethical Standards Regulations

Note the following options have been proposed for modification of CIRM MES Regulations 100070(f). Each could be noticed for public comment.

(f) CIRM-funded research introducing cells from covered stem cell lines into a live born human may not commence without SCRO committee review and approval in writing. The designated SCRO committee may require that modification be made to proposed research or documentation of compliance with the requirements of subdivision (f)(4) of this regulation as a condition of granting its approval. At a minimum, the SCRO committee shall ~~require the investigator to:~~
(1) ~~Provide~~ Confirm there is an acceptable scientific for rationale introducing stem cells into humans.
(2) Provide assurance that all covered stem cell lines have been acceptably derived.
(3) ~~Evaluate~~ Confirm the probable pattern and effects of differentiation and integration of the human cells into the human tissues have been evaluated.
(4) Provide documentation of compliance with any required review of the proposed research by an IRB, IACUC, IBC, or other mandated review.

Alternative:

CIRM-funded research introducing cells from covered stem cell lines into a live born human may not commence without IRB review and approval. The IRB must assure that in has adequate expertise to:
(1) Confirm there is an acceptable scientific for rationale introducing stem cells into humans.
(2) Provide assurance that all covered stem cell lines (as defined by CIRM regulation) have been acceptably derived.
(3) Confirm the probable pattern and effects of differentiation and integration of the human cells into the human tissues have been evaluated.
(4) Provide documentation of compliance with any required review of the proposed research by an IRB, IACUC, IBC, or other mandated review.

Note in text:
The IRB may choose a number of options, including asking a SCRO to review and approve the study, adding ad hoc members with appropriate expertise, or assuring its standing members provide needed expertise to comply with items 1 and 3 above.

DISCUSS Project: Considerations for SWG

- SWG has provided recommendations for prospective consent for iPSC derivation, banking, and distribution protocols. These recommendations have been incorporated into a model informed consent template.

- The DISCUSS project makes a good faith effort to apply the existing CIRM standards to research specimens not specifically obtained under a banking and distribution protocol.

- The CIRM iPSC Repository may accept cell lines derived from a variety of sources, so the DISCUSS points to consider may inform acceptance criteria.

- CIRM would the SWG review the points to consider.

- In particular, statements #2, #4, and #7 provide more specific guidance than the CIRM regulations.

- CIRM requests the SWG endorse the existing points to consider or provide recommendations for their modification.

DISCUSS Project: Timeline & Plan

- October 2013: Present & discuss with CIRM-SWG
- October 2013: Present & discuss to members of International Stem Cell Forum
- November 2013: Present & discuss at Public Responsibility in Medicine & Research (PRIM&R) annual meeting
- December 2013: Present & discuss with Interstate Alliance on Stem Cell Research / World Stem Cell Summit
- March 2014: Discuss and consider comments at DISCUSS Workshop involving international stakeholders
- Draft final recommendations