



M E M O R A N D U M

March 3, 2014

From: Ellen G. Feigal, M.D., Senior Vice President, Research and Development;
Elona Baum, Esq., General Counsel and Vice President, Business Development;
Ingrid Caras, Ph.D., Senior Science Officer

To: Independent Citizens Oversight Committee (ICOC)

Subject: Concept proposal for RFA 14-03: Strategic Partnership IV Agenda Item #6

This concept proposal addresses the continuation of CIRM's Strategic Partnership Initiative which was created to attract industry engagement and investment in CIRM-funded stem cell research. The intent of the Strategic Partnership program is to create incentives and processes that will: (i) enhance the likelihood that CIRM-funded projects will obtain follow-on funding for Phase 3 clinical trials; (ii) provide a source of co-funding in the earlier stages of clinical development, and (iii) enable CIRM-funded projects to access development expertise within pharmaceutical and large biotechnology partners.

The Strategic Partnership initiative is aligned with CIRM's 5 year strategic goal to attract industry engagement and investment in CIRM funded stem cell research and with CIRM's 5 year strategic clinical objective to advance stem cell science into clinical trials to achieve therapeutic benefit for patients.

Background:

The Strategic Partnership concept was approved by the ICOC on October 26, 2011 and amended on September 5, 2012 and on October 25, 2012. The current concept directs CIRM to implement the program using a standard Request for Application (RFA) process that has a defined submission period. Each RFA will define the specific scope of research to be funded, within the currently approved concept scope which encompasses a broad range of research from basic research to Phase 2 clinical studies.

Specific features of the Strategic Partnership concept include (i) a requirement that the applicant show evidence of either having the financial capacity to move the project through development or of being able to attract the capital to do so (Commercial Validation) and (ii) a requirement to provide co-funding for the proposed project.

To date, there are two active awards under the Strategic Partnership concept, one under Strategic Partnership I and one under Strategic Partnership II. Strategic Partnership III recommendations will be brought to the ICOC on May 29, 2014.

Under the current Strategic Partnership IV concept plan, CIRM will fund up to \$10M justifiable

total project costs per project, with the possibility to increase the award up to \$12M per project, subject to ICOC approval and only under exceptional circumstances.

Active management of these milestone-driven projects will include setting mutually agreed-to milestones prior to initiating the award and discussion with CIRM's external Clinical Development Advisory Panel (CDAP) annually or at key decision points.

Concept Proposal for RFA 14-03: Strategic Partnership IV

RFA 14-03, the fourth RFA under the Strategic Partnership initiative, is similar to Strategic Partnership I, II and III in objective and other key aspects, but is focused on clinical stage projects that have filed an IND and limits the project period to 3-years.

Award: CIRM will fund up to \$10M/award over up to 3-years for 1 project (\$12M in extraordinary circumstances).

Objective: Completion of a Phase 1 or Phase 2 clinical trial within the 3-year time frame.

Eligibility: Requires Commercial Validation.

Co-funding: Requires co-funding from the applicant

Readiness: IND has been submitted

Award information:

Total allocation for SPIV - \$32M

Award mechanism:

Grant, if PI holds the IND and is from a not-for-profit organization

Choice of Grant or Loan, if a for-profit organization holds (sponsors) the IND and is the applicant organization.

Provisional Time Table:

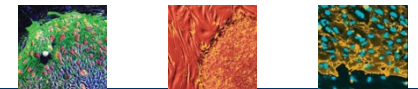
Post RFA 14-03	April	2014
LOI applications due	Q2	2014
Full Applications due	Q3	2014
Review of Applications by Grants Working Group (GWG)	Q4	2014
Review and Approval by ICOC	Q1	2015
Earliest Funding of Awards	Q2	2015



Consideration of Strategic Partnership IV Concept Plan

Pre-Read
for
ICOC Meeting March 13, 2014
Agenda Item # 6

Ingrid Caras, M.D.
Senior Science Officer



Presentation Outline

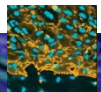
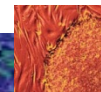
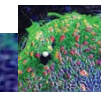


This Concept Proposal addresses the continuation of CIRM's
Strategic Partnership Initiative

- Approved by the ICOC in Oct 2011

Goals:

- Review what the Strategic Partnership Initiative is about
 - Purpose and objectives
- Present concept plan for **Strategic Partnership IV**
 - The fourth call under the Strategic Partnership Initiative



Purpose: Strategic Partnership Initiative



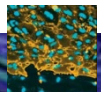
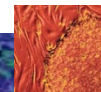
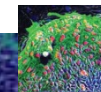
- Purpose of the SP Initiative is to attract **industry engagement and investment** in CIRM-funded stem cell research
- To
 - (i) provide a source of **co-funding** in the early stages of development
 - (ii) enable CIRM-funded projects to access **development expertise** within large pharma/biotech partners
 - (iii) enhance the likelihood that CIRM-funded projects will obtain **follow-on financing** for late-stage development and commercialization



Alignment with Strategic Plan



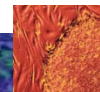
- **SP Initiative** is aligned with the Clinical and Economic objectives of CIRM's **Strategic Plan**
- Aligned with CIRM's **5 year strategic goal** to attract industry engagement and investment in CIRM funded stem cell research
- Aligned with **Clinical strategic objective** to advance stem cell science into clinical trials to achieve evidence of therapeutic benefit to patients



Objective: Strategic Partnership IV



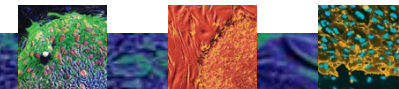
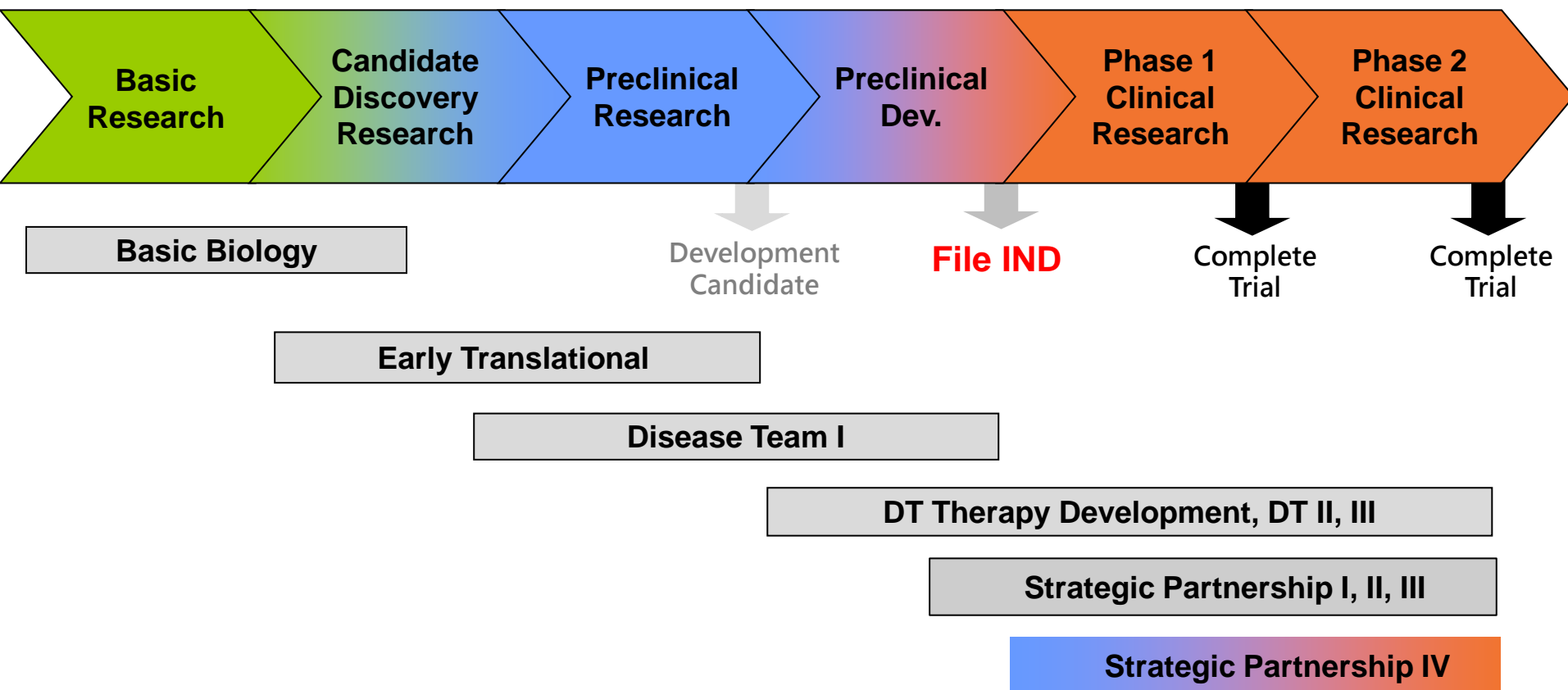
- Objective of a Strategic Partnership IV Award will be to complete a Phase 1 or Phase 2 **Clinical Trial** within 3-years



Scope: Strategic Partnership IV



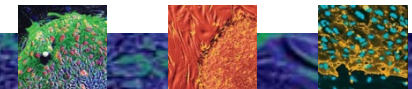
SPIV is designed to capture **mature** programs already at an **Early Clinical Development stage**



Status Update of SP Awards



- **Viacyte - SPI Award**
 - To complete a Phase 1 trial of hESC-derived pancreatic cells in a device, for **Type 1 Diabetes**
- **Sangamo - SPII Award**
 - To file IND and complete a Phase 1 trial for genetically engineered blood stem cells to treat a blood disorder, **beta-thalassemia**
- **SPIII** recommendations will be brought to ICOC May 2014
- Jan 2014: **Biogen Idec** and **Sangamo** announced Global Collaboration
 - To co-develop treatments for **beta-thalassemia and sickle cell disease**

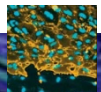
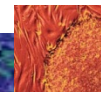
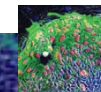


Unique Features: Strategic Partnership Initiative



1. **Requires** applicants to show they have **financial capacity** to move the project through development or are able to attract the capital to do so
 - Evidenced by 1 or more of the following:
 - (i) investments from VC firms, public markets, pharma/biotech companies, disease foundations
 - (ii) significant Liquid Assets
 - (iii) a Research/Development agreement with a large pharma/biotech company

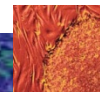
2. **Requires** applicants to provide **co-funding** for proposed project - 1:1 match



Eligibility: Strategic Partnership IV



- Open to for-profit and not-for-profit institutions
- Focused on mature **Clinical Stage** projects
 - IND filed

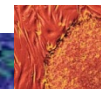


Commercial Validation: Strategic Partnership IV



All applicants must provide evidence of commercial validation

- **For-profit applicants**
 - either by financial strength
 - and/or via a research/development agreement with a large biotech/pharma partner
- **Not-for-profit applicants**
 - must have a research/development agreement with a large biotech/pharma partner



Activities: Strategic Partnership IV

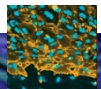
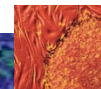
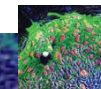


In-Scope

- Conduct of early clinical trials
Phase 1 or Phase 2
- Supporting activities such as
manufacture of product

Out of Scope

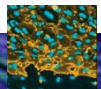
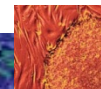
- IND-enabling activities
- Phase 3 trials



Award Information: Strategic Partnership IV



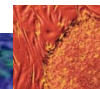
- **SP IV TOTAL COSTS**
 - Up to \$32M
 - Up to 3 awards
- **Amount**
 - \$10 M per project
 - Under exceptional circumstances up to \$12M
- **Award Term**
 - 3-years
- **Co-funding**
 - 1:1 match
- **Award mechanism**
 - Grant if not-for-profit organization
 - Loan or Grant if for-profit



Management & Review Process Post Funding-Approval



- Prior to grant initiation CIRM Science Officer and Awardee set mutually agreed-to **Milestones**
 - including key Go/No Go decision points and criteria
- Active management of funded development projects
 - includes assessment by CIRM's external Clinical Development Advisory Panel (**CDAP**) annually or at key decision points



Strategic Partnership IV

Provisional Time Table



SP IV Provisional Time Table		
Post RFA 14-03	April	2014
LOI applications due	Q2	2014
Full Applications due	Q3	2014
Review of Applications by Grants Working Group (GWG)	Q4	2014
Review and Approval by ICOC	Q1	2015
Earliest Funding of Awards	Q2	2015

