ICOC Meeting

Presentation of the Draft Scientific Strategic Plan

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President

Development of the Scientific Strategic Plan

- October, 2005: *Stem Cell Research in California: Charting New Directions*
- April, 2006: A “Plan for a Plan”
- April, 2006: Engaged PwC as consultants

Development of the Scientific Strategic Plan (cont.)

- Interviewed over 70 scientists, clinicians, ethicists, patient advocates, public interest representatives
- Held three public meetings for the ICOC and the public
- Held two focus groups: Patient Advocates and Diversity
- Two ICOC meetings focused on Mission Statement; Values; Strategic Principles
- Seven Strategic Plan Advisory Committee meetings

Strategic Plan Advisory Committee

- David Baltimore, Cal Tech
- Paul Berg, Stanford
- George Daley, Harvard Medical School
- Steve Forman, City of Hope
- Zach Hall, CIRM (Chair)
- Sherry Lansing, ICOC
- Robert Klein, ICOC
- Ed Penhoet, ICOC
- Bill Rastetter, past CEO of Biogen-IDEC
- Jeff Sheehy, ICOC

Strategic Plan Team

Patricia Olson, CIRM
Tony Pillari, PwC

Raymond Anderson, PwC
Arlene Chiu, CIRM
Gil Sambrano, CIRM
Mary Maxon, CIRM
Amy Lewis, CIRM
Kate Shreve, CIRM
Pat Becker, CIRM
Christine Woo, PwC

Gerry McDougall, PwC
Bill Dracos, PwC

Scientific Strategic Plan

- Executive Summary, Body of Report, Appendices
- Receive input from the ICOC at the October meeting, particularly on objectives, general direction, emphasis
- Receive input from others
- Modify and present to the ICOC for approval in December

Strategic Plan Goals

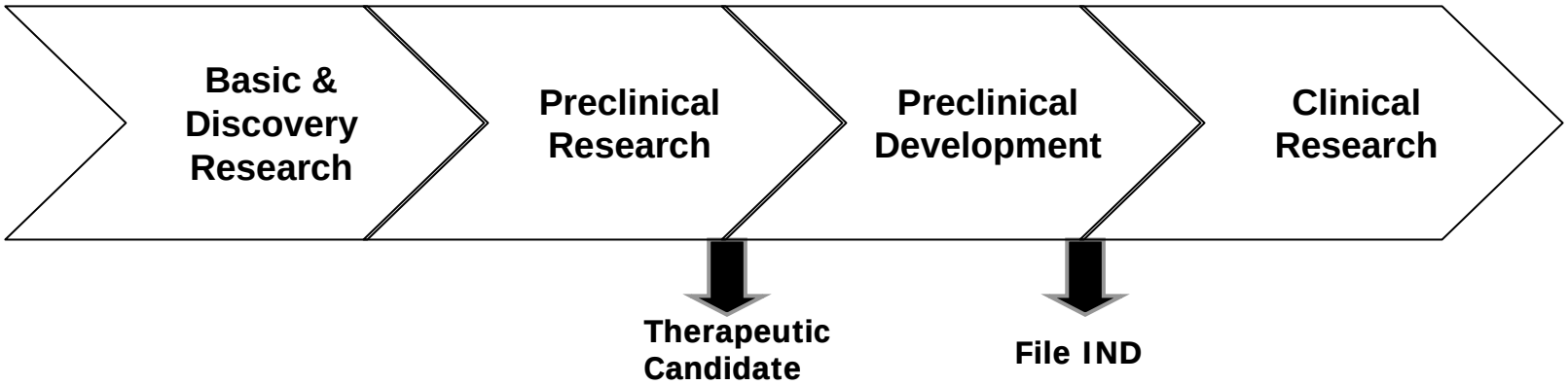
- **<u>Aspirational Goals</u>**:
  - *What we dream to achieve*
  - *Cure disease*
  - *California as world-wide leader in stem cell research*
- **<u>Commitment Goals</u>**:
  - *Our covenant with the people of California for what we will achieve over the next ten years to make the promise of stem cell research a reality*

Commitment Goals

- Focused on human embryonic stem cells, with emphasis on cell replacement therapy
- Realistic goals for which we will be accountable
- Set ten year goals, then set five year goals as milestones against which to measure our progress

Cell Therapy Development

Stages of Cell Therapy Development



Clinical Trials

Phase	Purpose	Number of Patients
Phase I	Safety	Tens
Phase II	Dose, regimen, efficacy signal; safety	Tens to hundreds
Phase III	Statistical proof of efficacy; safety	Hundreds or more

Lessons from Small Molecule and Biological Therapeutic Development

- On average, 7-9 years from the start of clinical development to approval for the market
  - Thus, we are unlikely to bring a therapy all the way to market within ten year plan
- Attrition at every stage of development: only 1 in 8-10 therapeutics that enter clinical development are approved for the market
  - Thus, need a strong pipeline that will continue to bring products into the clinic past the ten year period of plan.

Stem Cell Therapeutics

- Human embryonic stem cell research a young, but growing field.
  - from 1998 to end of 2004, only 132 publications
  - rapid growth, but still much to learn
- Cell replacement therapy a new therapeutic modality
  - BMT and fetal transplants involve minimal manipulation

Ten Year Goals

- *Goal 1*: Clinical proof of principle that transplanted cells derived from pluripotent cells can be used to restore function for at least one disease.

- *Goal 2*: Therapies based on stem cell research in Phase I or Phase II clinical trials for 2-4 additional diseases

Ten Year Goals (cont.)

- *Goal 3*: Attract private capital for Phase III trials
- *Goal 4*: New approaches for achieving immune tolerance
- *Goal 5*: Proof of principle for therapies in preclinical models for 6-8 diseases
- *Goal 6*: Create and use disease-specific lines for 20-30 diseases.

Ten Year Goals (cont.)

- *Goal 7*: New procedures for large-scale GMP production of stem and progenitor cells.
- *Goal 8*: A thorough understanding of steps of stem cell differentiation.
- *Goal 9*: A thorough understanding of factors regulating self-renewal and oncogenic potential of stem cells.
- *Goal 10*: New methods of tissue replacement based on stem cell research.

Five Year Goals

- *Goal 1*: Six therapies based on stem cell research in pre-clinical development.
- *Goal 2*: New methods of making stem cell lines.
- *Goal 3*: Disease-specific cell lines for four diseases
- *Goal 4*: Methods of growing hSCs in defined media
- *Goal 5*: Establishment of a stem cell bank.

Five Year Goals (cont.)

- *Goal 6:* Demonstration of immune tolerance in animal models.
- *Goal 7*: Increased workforce of stem cell researchers in California.
- *Goal 8*: Toxicity testing based on stem cell research.
- *Goal 9*: Effective partnerships between non-profit and commercial sectors.
- *Goal 10*: National and international collaborations.

Strategic Plan Framework

- Initiatives represented within two-dimensional “space”
- Horizontal axis is progress from fundamental to clinical research
- Vertical axis represents the kinds of resources that we will use

Strategic Planning Framework

Laying the Foundation → **Preparing for the Clinic** → **Clinical Research**

Scientific Training & Development

Innovation Science

Mission-Oriented Science

Tools, Technologies & Infrastructure

Facilities

Communities of Science

Responsibility to the Public

Initiatives

Initiatives Overview

- Topics based on 2005 scientific meeting, interviews, scientific meetings for ICOC and the public
- More detailed for earlier initiatives (fundamental/preclinical research) than on later (preclinical and clinical development)
- Dollars based on estimate of how many grants, how large, how many years.
- Initiatives are not final: each RFA, including budget, approval will come to ICOC for approval
- Workshops often used to gather further information
- Priorities, topics and budgets may change

Overview of Initiatives

- Scientist Training / Internships
- Technical Staff Training
- Scientific Personnel Development
- hESC Jump Start Initiative
- Annual Innovation Grants
- Biology of Stem Cells
- Egg and Embryo Research
- New Methods for Development of Stem Cell Lines
- Stem Cell Based Tissue Engineering in Regenerative Medicine
- Translational Research
- Generation and Use of Disease Specific Cell Lines
- Immune Tolerance
- Bio-Process Engineering and Automation
- Preclinical Product Development
- Clinical Investigation
- Disease Teams
- Interdisciplinary Research Teams
- Tools and Technologies
- Cores
- Banks
- Laboratories / Research Facilities
- Journal / Web Portal
- Public Outreach
- Stem Cell Research and Society: Implications and Impact
- Economic Impact

Special Programs Initiative

- Exploration of a different mechanism of organization
  - Collaborative teams across institutions
  - Specific goals with a timeline and milestones
  - Active project management
  - CIRM participation in evaluation and management
- Disease Teams
- Research Teams

Budget

- Estimated how much money coming in from bond issuance each year (Appendix 3)
- Estimated budgets for activity (workshops, RFA) based on how many years, how many grants, how much per grant
- Drew up year-by-year plan (Appendix 4)
- Opportunity funds
- Analysis by “Pathway to the Clinic”

Overview of Funding Estimates

Estimates for funding totals for each resource category are anticipated to be as follows:

	Laying the Foundation	Preparing for the Clinic	Clinical Research	Total
Research Activities				
Scientific Training and Development	$ 165.5	$ 64.7	$ 64.7	$ 295.0
Innovation Science	$ 263.3	$ 56.8	$ 56.8	$ 376.8
Mission-Directed Science	$ 226.0	$ 585.0	$ 461.1	$ 1,272.1
CIRM Special Programs	$ 48.4	$ 72.8	$ 60.8	$ 182.0
Tools / Technologies and Infrastructure	$ 107.0	$ 107.0	$ -	$ 213.9
Communities of Science	$ 1.9	$ 1.9	$ 1.9	$ 5.6
Responsibility to the Public	$ 10.8	$ 10.8	$ 10.8	$ 32.3
Totals	**$ 822.8**	**$ 898.9**	**$ 656.0**	**$ 2,377.7**

Facilities				
Facilities	$ 190.9	$ 81.8	$ -	**$ 272.7**
Totals	**$ 190.9**	**$ 81.8**	**$ -**	**$ 272.7**

A Living Plan

- Review at years 3 and 7 by a blue-ribbon, outside committee which will make recommendations for modification and adjustment of goals, objectives, initiatives
- CIRM may also wish to sponsor conference
- ICOC will consider recommendations of the Review Committee and approve modification.
- CIRM President and Staff will convert into an operational plan for ICOC approval.

Next Steps

- Will hear your suggestions and those of others
- Make modifications in Plan
- Add section on First 1000 Days
- Bring final document to ICOC for consideration, modification and approval in December.