CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE

The state stem cell agency

Development Portfolio Review

June 2010

Patricia Olson, Ph.D.
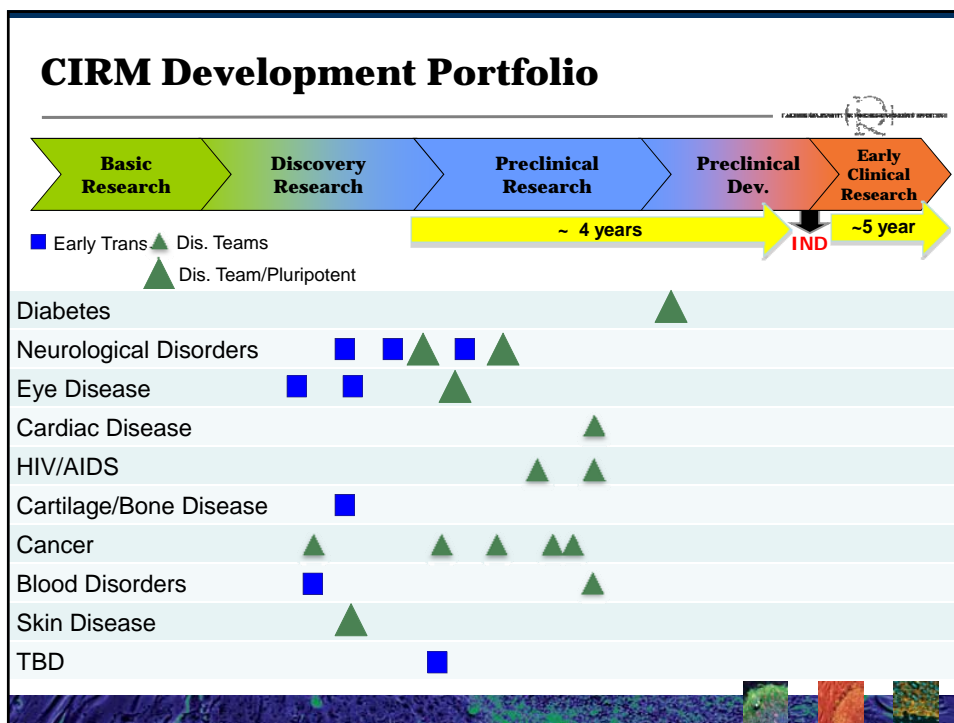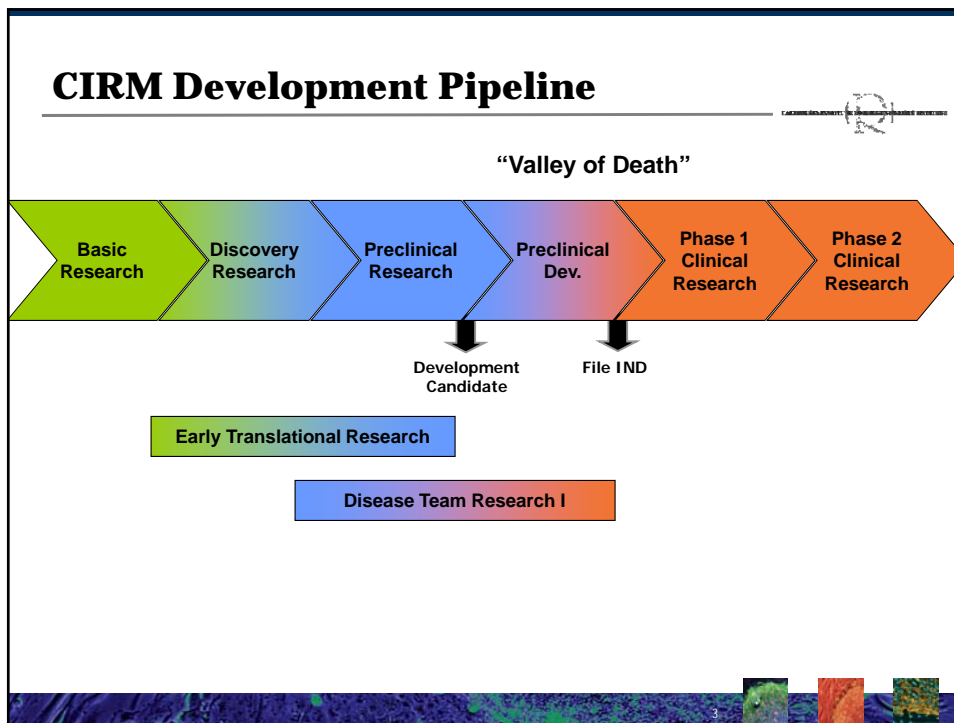*Executive Director, Scientific Activities*
Agenda Item # 16

6/7/10

Objectives

- Provide an overview of our current development portfolio
- Facilitate programmatic decision making

CIRM Development Pipeline

"Valley of Death"

| Basic Research | Discovery Research | Preclinical Research | Preclinical Dev. | Phase 1 Clinical Research | Phase 2 Clinical Research |

Development Candidate

File IND

Early Translational Research

Disease Team Research I

CIRM Development Portfolio

| Basic Research | Discovery Research | Preclinical Research | Preclinical Dev. | Early Clinical Research |

~ 4 years   IND   ~5 year

■ Early Trans ▲ Dis. Teams
▲ Dis. Team/Pluripotent

Diabetes
Neurological Disorders
Eye Disease
Cardiac Disease
HIV/AIDS
Cartilage/Bone Disease
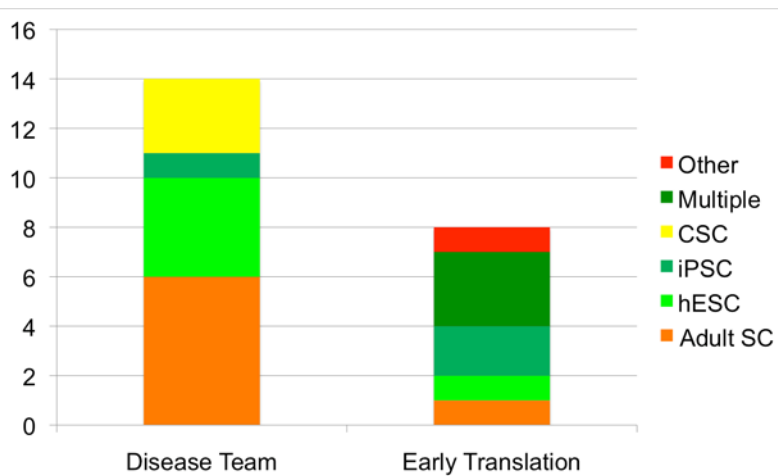Cancer
Blood Disorders
Skin Disease
TBD

Development Portfolio: Stem Cell Source or Target

Awards from Disease Team I & Early Translation I RFAs
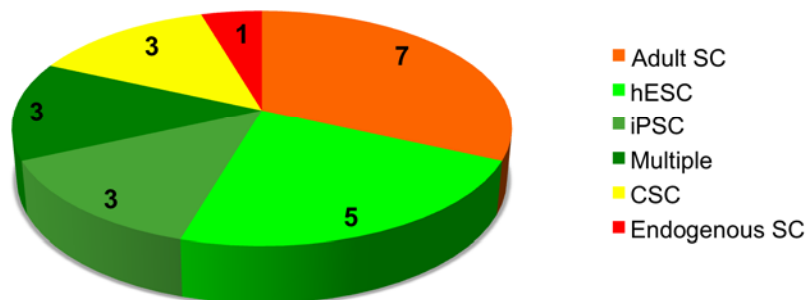
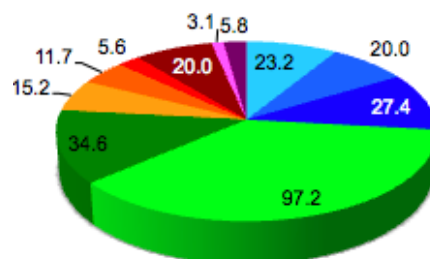## Development Portfolio: Stem Cell Source/Target

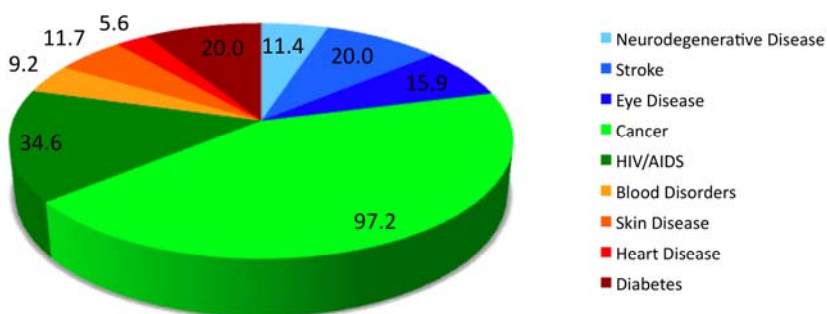# Development Portfolio: Disease Distribution

Disease Team Awards Portfolio: Investment

Development Portfolio: Therapeutic Approach

Awards from Disease Team & Early Translation RFAs

Legend:
- Cell Therapy
- Genetically Modified Cell Therapy
- Cell Therapy Combination Product
- Protein Combination Product
- Drug/Monoclonal Antibody
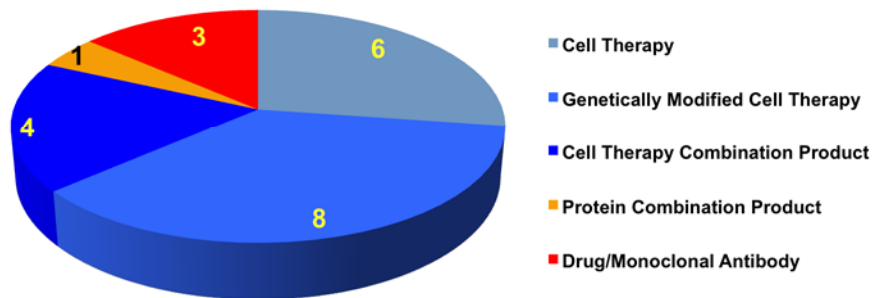
Portfolio Considerations

- Strategic/Portfolio Focus
- Regulatory Risk

Lower → Higher

| Small Molecule, MAb, Protein | Adult Stem Cell | Gene-Modified Adult Stem Cell | Pluripotent Stem Cell-Derived Cell | Gene-Modified Pluripotent Stem Cell-Derived Cell |
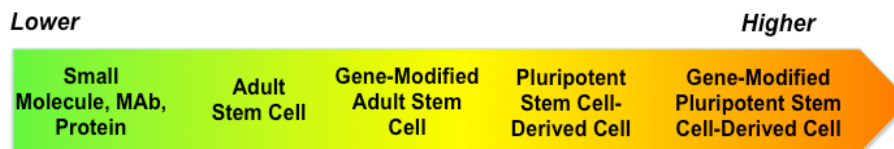
- Business model to bring to patients

CIRM Development Portfolio: Approach (1)

Legend: ☐ Early Translational — ■ Allogeneic (red) ☐ Autologous (yellow) ■ Other (magenta); △ Disease Teams — ▲ Allogeneic (red) △ Autologous (yellow) ▲ Other (magenta)

	Small Molecule, MAb, Protein	Adult Stem Cell	Gene-Modified Adult Stem Cell	Pluripotent Stem Cell-Derived Cell	Gene-Modified Pluripotent Stem Cell-Derived Cell
Diabetes				▲ (red)*	
Neurological Disorders					
ALS				▲ (red)	
Stroke				▲ (red)*	
AD				■ (red)	
HD			■ (red)		
PD		■ (red)		Or ■ (red)	Or ■ (red)
Eye Disease: AMD				▲ (red)*	☐ (yellow)
"		☐ (yellow)	Or ☐ (yellow)		Or ☐ (yellow)
Cardiac Disease		△ (yellow)			

* Combination product

CIRM Development Portfolio: Approach (2)

Legend: ☐ Early Translational — ■ Allogeneic (red) ☐ Autologous (yellow) ■ Other (magenta); △ Disease Teams — ▲ Allogeneic (red) △ Autologous (yellow) ▲ Other (magenta)

	Small Molecule, MAb, Protein	Adult Stem Cell	Gene-Modified Adult Stem Cell	Pluripotent Stem Cell-Derived Cell	Gene-Modified Pluripotent Stem Cell-Derived Cell
Cartilage Disease				■ (red)	Or ■ (red)
HIV/AIDS			△ (yellow)		
"			△ (yellow)		
Cancer					
Hematologic	△ (magenta)				
"	△ (magenta)				
Solid Tumor	△ (magenta)		▲ (red)*		
"			▲ (red)		Or ▲ (red)
Blood Disorders			△ (yellow)		☐ (yellow)
Skin Disease					△ (yellow)
TBD (Stroke, Heart Disease, Skin Ulcers, Fractures)	■ (magenta)				

*Combination product