

# California Institute for Regenerative Medicine

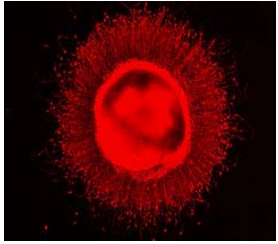
---

*#1 Overview*

## External Review

***Alan Trounson***  
***President***

[www.cirm.ca.gov](http://www.cirm.ca.gov)

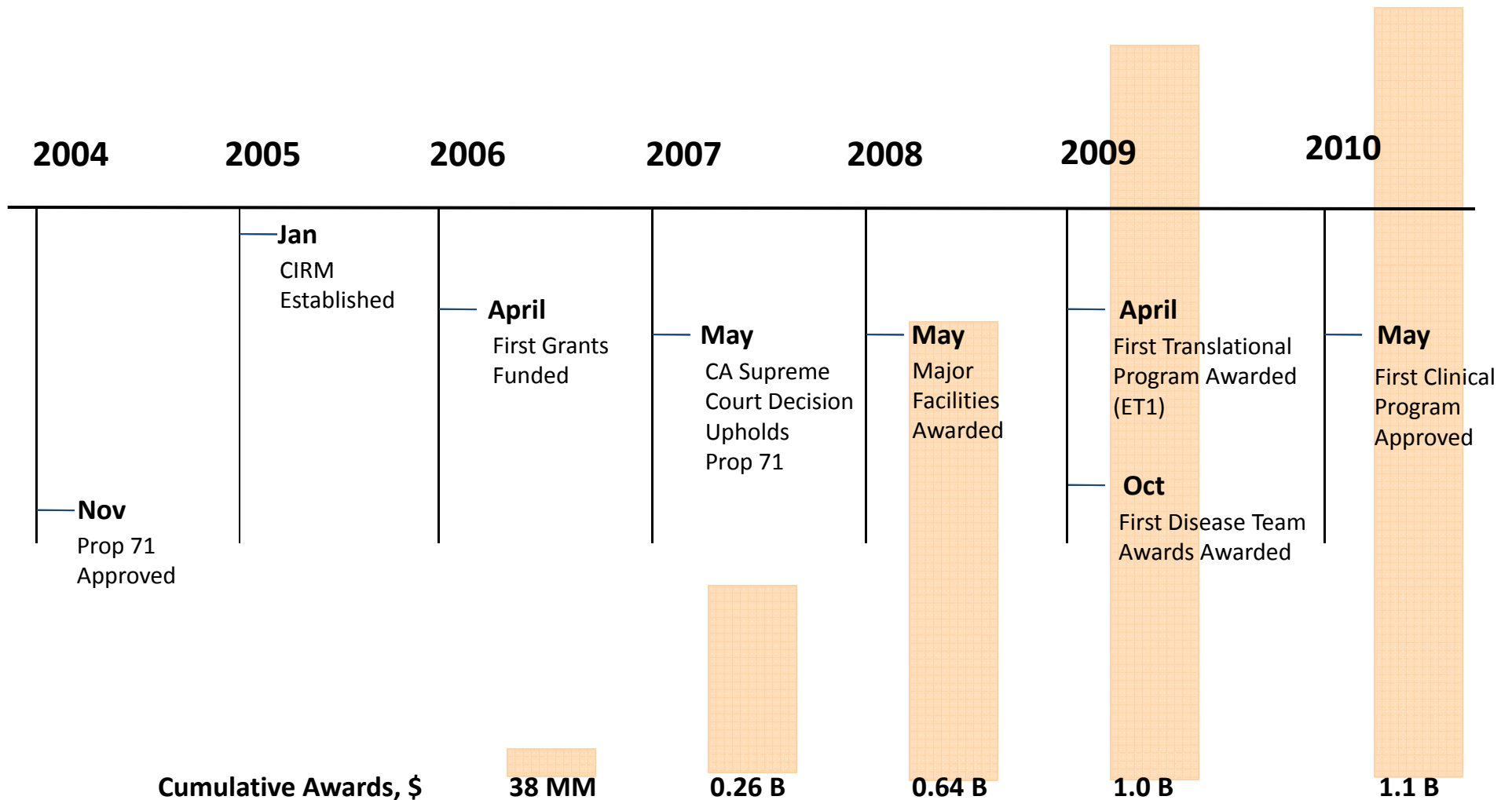


# Purpose of Review Process

Formal assessment by an outside panel of the 2006 plan was recommended at years three and seven. Year 1 for the plan was designated to start July 1, 2007

- Goals of the evaluation process are:
  - Evaluate CIRM's programs against its goals
  - Assess effectiveness in moving CIRM towards meeting its goals and accomplishing its mission
  - Recommend changes in CIRM's funding priorities to ensure that CIRM is supporting the most promising advances in the field of regenerative medicine
  - Other feedback as necessary

# CIRM Timeline



# Proposition 71

- Approved by 59% of CA voters (2004)
- Authorized \$3 billion of State Obligation Bonds to fund stem cell research in CA (max \$300mill/yr) **<6% for admin.**
- Established a 29 member Board that meets in public with CIRM Management (>8 times/yr) – all final funding decisions made by Board
- Required development of medical and ethical standards

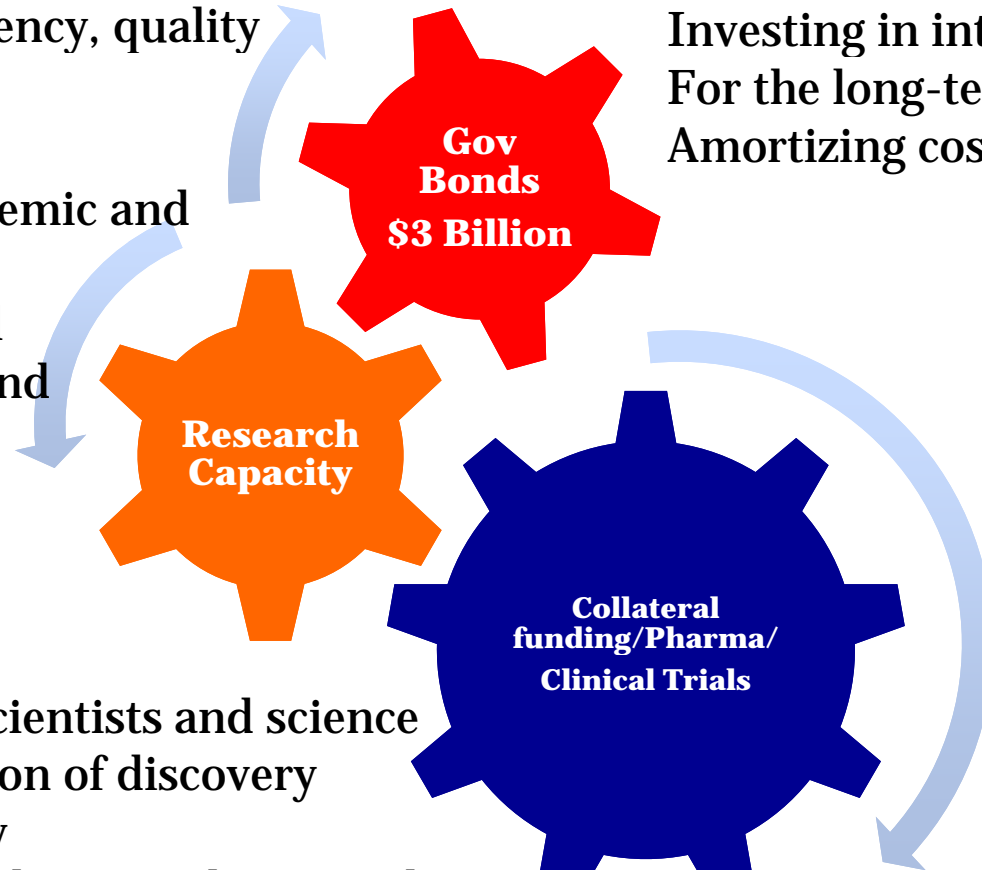


# California Institute for Regenerative Medicine

Community support, industry and academia support, patient advocate partnership, transparency, quality

Stimulus to Cal. academic and biotechnology sector  
Building institutional research excellence and collaborations

Supporting the best scientists and science  
Encouraging translation of discovery to clinical opportunity  
International partnerships – enhancing the best - critical to success



Investing in intellectual capital  
For the long-term  
Amortizing costs across benefits

Economic benefits of patient cures and quality of life, reduced health care costs, commercialization concurrent benefit but not sole driver

# Strategic Approach

## Mission

Establish California as a World Class Leader in Regenerative Medicine by Building Infrastructure, Training Our Future Scientists & Funding Research Having the Greatest Potential to Yield Therapies Which Improve Patients' Lives & Relieve Suffering

## Strategic Focus

**Access to  
Most  
Promising  
Research**

**Leadership -  
Elimination  
of Barriers**

**Infrastructure  
(Physical,  
Intellectual)**

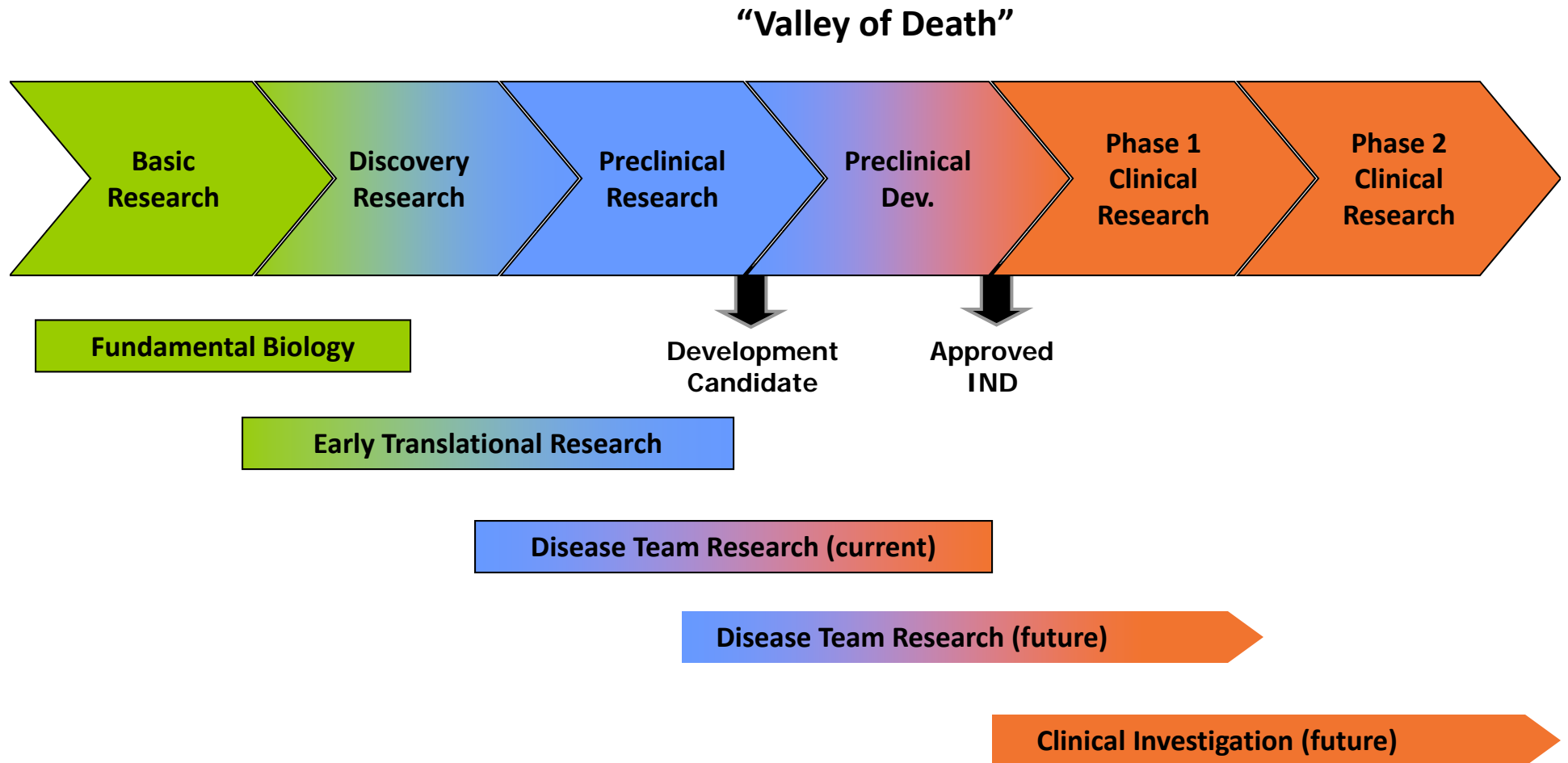
## Initiatives

- Collaborative Funding Partners
- Academic / Comprehensive / Seed/ Basic Grants

- Leadership Awards
- Team Building
- Major Facilities
- Regulatory Outreach
- Industry Engagement

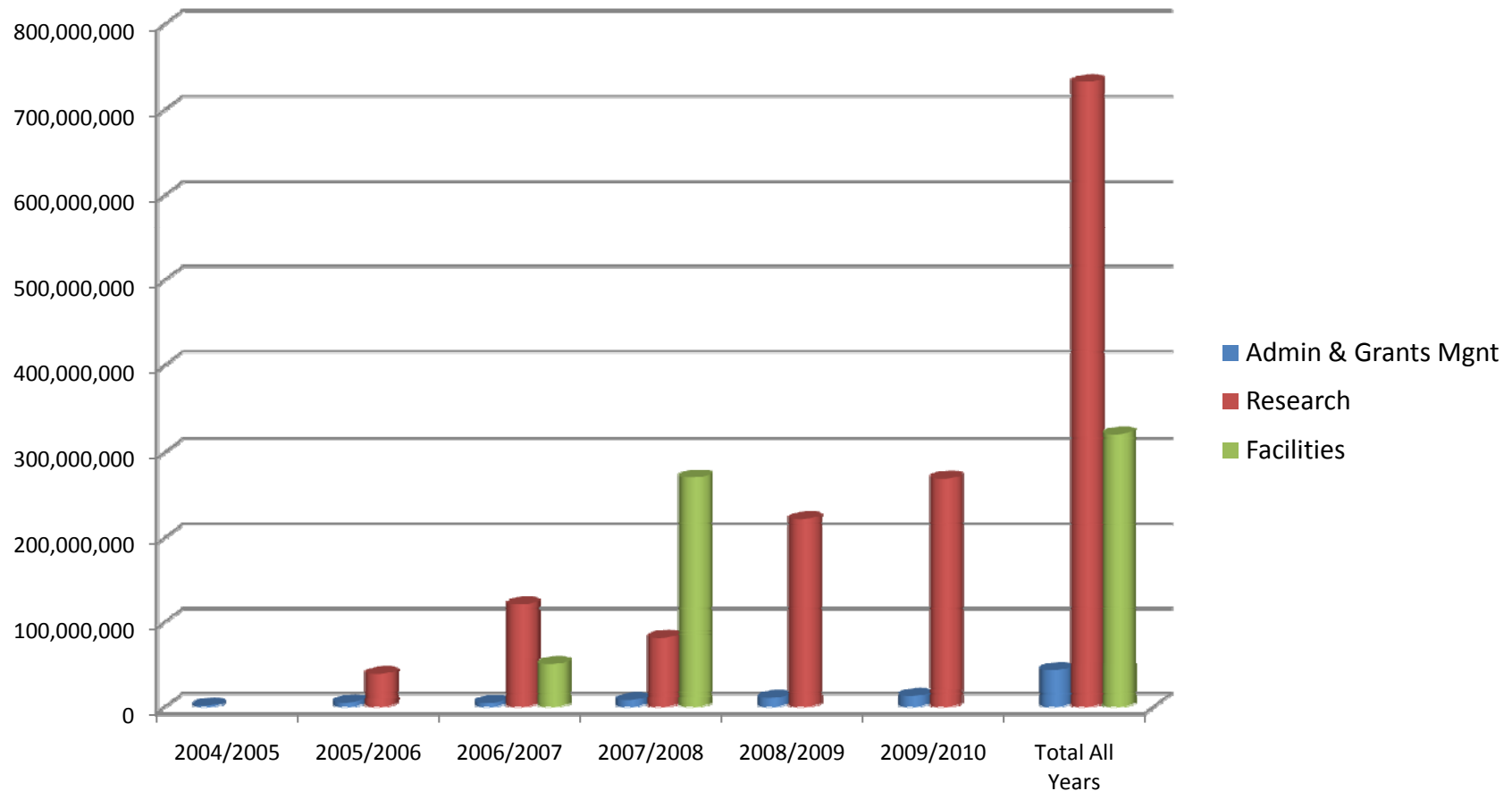
- Fellows Program
- Training Grants
- Bridges
- Shared Facilities
- Tools & Technologies

# Core Programs: Sustaining a Pipeline



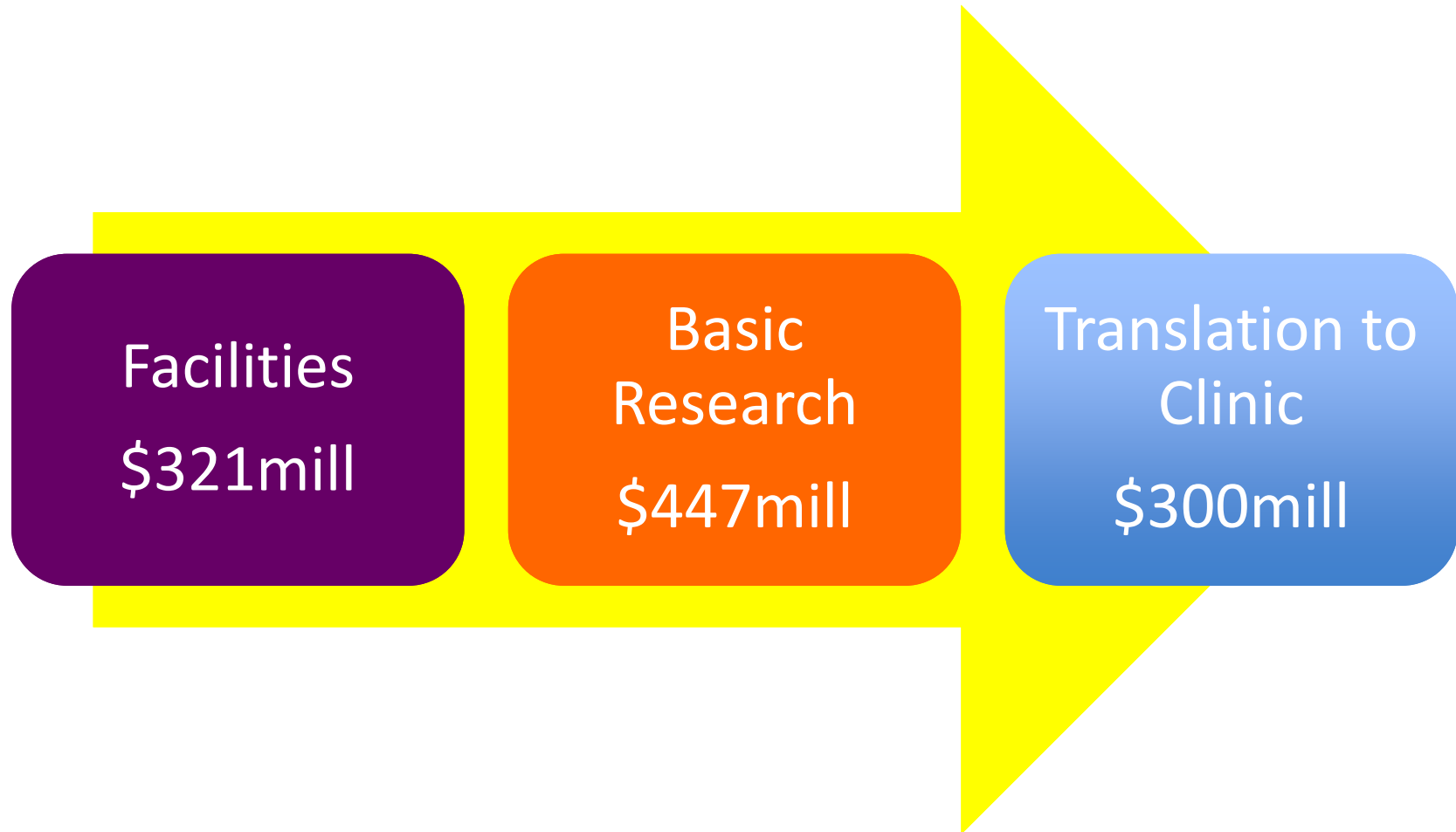
# **A New Institute – Allocation of funds – June 2010**

## **Max 6% on admin and grants management**

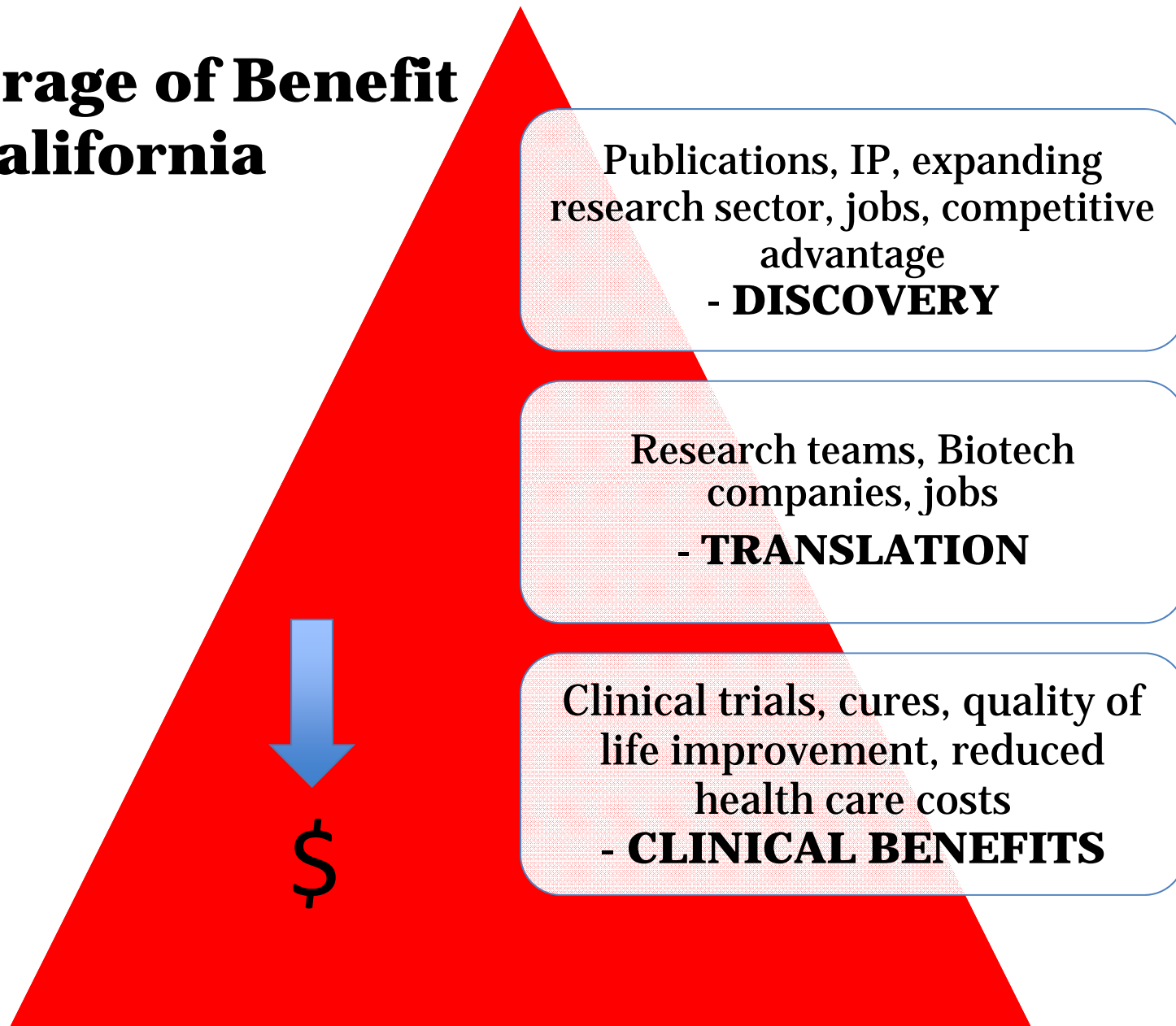


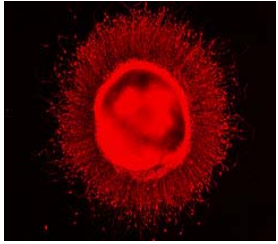


# Where are we in the pipeline?



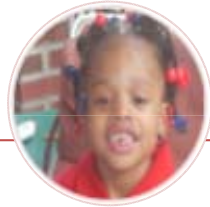
# Leverage of Benefit To California





# Major Milestones

- 364 research and facilities grants awarded
- 53 CA Institutes/Companies with CIRM grants
- 12 new institutes and centers of regenerative medicine ~\$1 billion (\$271mill from CIRM)
- \$1.07 Billion in grants allocated
- Over 600 major scientific papers published
- 102 new stem cell researchers in California
- Two clinical trials arising from CIRM funding
- 14 Disease Teams (preclinical) awarded – up to \$20mill grants aimed for IND (FDA) within 4yrs
- First Clinical RFA released for hESC derived therapy



**Discovery**

**Regulatory  
Pathway**

**Translation**



**α Stem  
Cell Clinic**

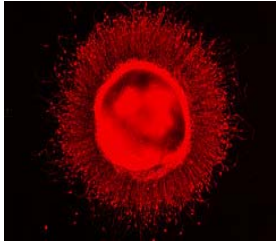


**Clinical Trials**



**Commercialization**





# **Vision**

- Maintain a strong discovery platform of basic research
- Training the next generation of stem cell scientists
- Link the best researchers globally with California
- Encourage team collaborations across institutes, state boundaries, public-private sectors
- Manage the portfolio to optimize outcomes
- Be open to new developments and embrace innovation with optimism
- Recognize excellence in basic and translational research and support their new directions



# CIRM Operational Excellence

**Engage with stakeholders and have their strong support**

Clarity of purpose

Transparency of decisions



**Openness and excellence**

Back the best proposals; be flexible

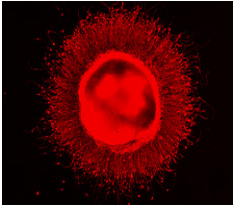
Be responsive and clear about direction



**Make smart decisions**

Benefits of cures – to patients and to  
State

Leverage investments and create returns



# Questions to consider

- **Is CIRM selecting proposals that will maximally accelerate the field?**
- **Does CIRM's Core Grant program offer a sufficient blend of predictability and flexibility to get the best proposals?**
- **Are we providing effective oversight to maximize the pace of success of individual projects and the synergy between projects?**
- **Which process improvements deserve the highest priority?**
- **Should CIRM speed up its rate of investment or reserve more funds to better take advantage of advances in the field?**
- **Is there something more CIRM should be doing to get effective handoff to industry?**
- **Should CIRM be doing more to assure Intellectual Property discovered with its funding is properly protected with patents?**
- **Which new initiatives under consideration (page 40 in Briefing) deserve highest priority?**
- **Which initiatives should CIRM engage in to mitigate potential challenges?**
- **Should CIRM seek added financing to extend its funding program?**

