

# The NIH Clinical IMPACT Program: Bench To Bedside And Beyond



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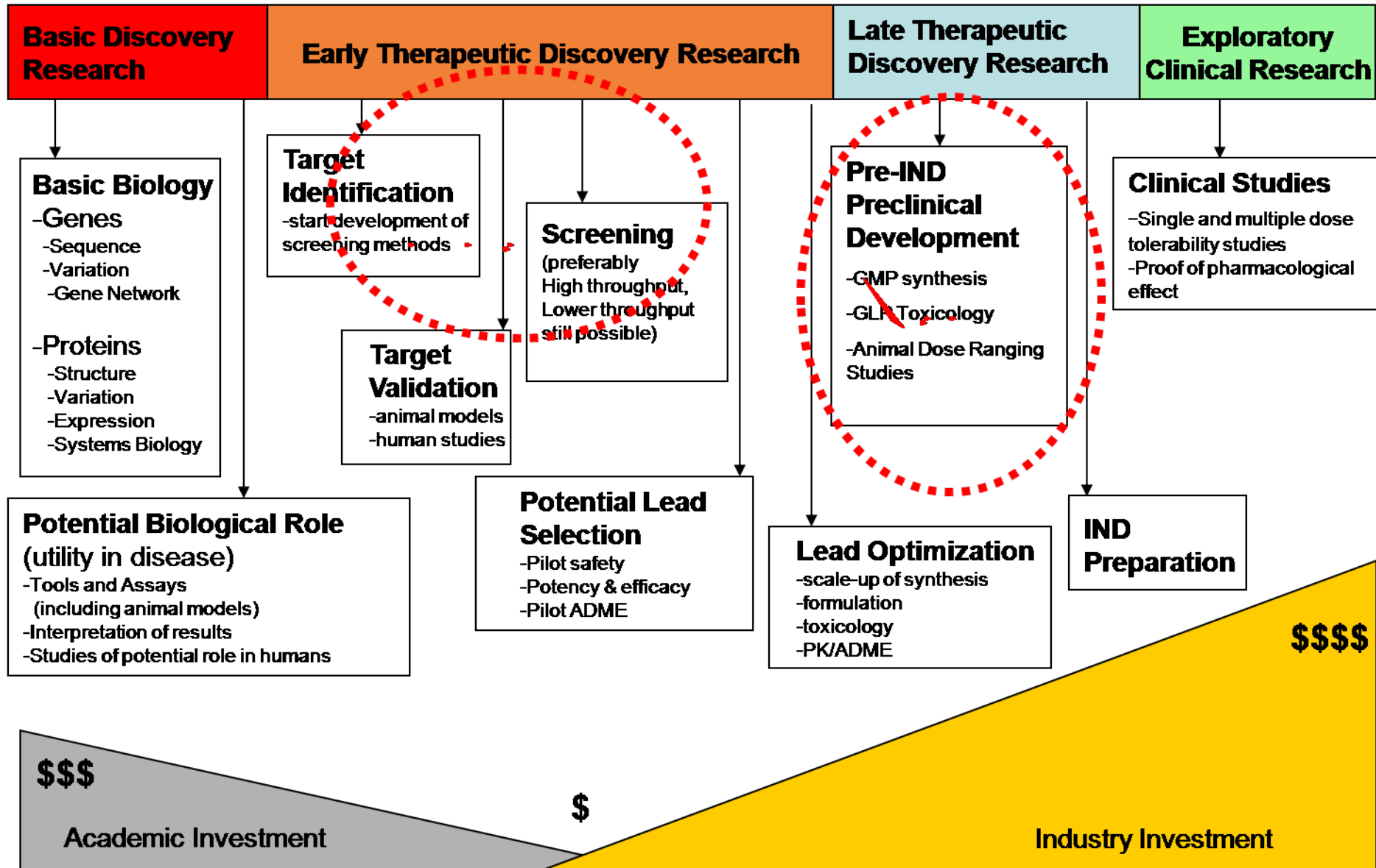
# Center for Cures Proposed in Congress 2005

Writing in Thursday's *Commercial Appeal of Memphis*, Harold Ford Jr. and Al From break that mold with some out-of-the-box thinking - which is always how real change starts. While giving a nod to the reform plans of their party's presidential candidates, the two leaders propose an "American Center for Cures" as a critical element in speeding the development of cures for human illness and injury. (May 8, 2008)

# Katie Hood, CEO, The Michael J. Fox Foundation for Parkinson's Research

“I would love to see the presidential candidates express their views on the concept of a Center for Cures and the potential for "progress-driven" reform of the health care system. After all, what good is it to tinker around the edges of the most expensive health care system in the world - or to fund the greatest discovery engine anywhere - if we're not converting all that investment into real improvements in human health?”

# The Path from Discovery Research to Bedside for New Therapeutics



# Impact Program

Catalyze translation of basic discovery to impact on human disease

Provide environment for transformation

Incentives to encourage risk

# Roadmap: creating new “Out of the box” mechanisms

Pioneer Awards- funding creative individuals

New Innovator Awards- selecting emerging pioneers

Transformative R01s- funding creative ideas

What these mechanisms have in common:

Encourage risk from proven performers

Outcome- New discovery

**Maximum of 5 years, nonrenewable**

What about the really big challenges like impacting human health?

Discovery is not enough.

Does limiting duration limit our ability to take on the most difficult problems?

Incentives must be sufficient to overcome fear of failure.

# Principles Of IMPACT Award

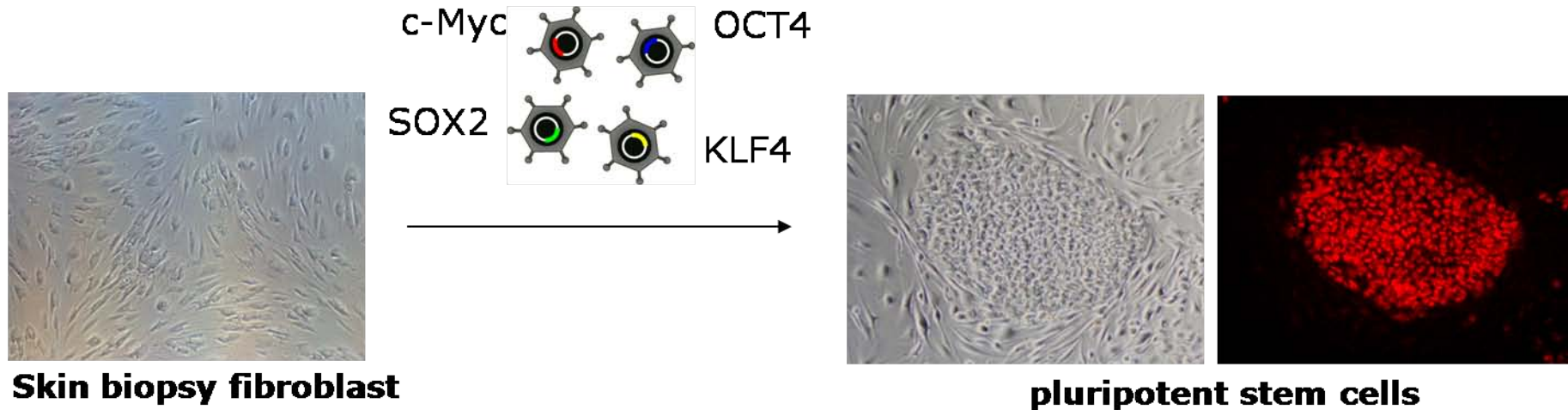
1. Major effort of PI(s)
  - Collaborative teams, flexible make-up
2. Potential for stable, longterm funding
  - 10 years plus without study section peer review
3. Interim review based on milestones
  - 2 or 3 phases with measurable milestones
  - External panel, focused on milestone achievement
4. Significant level of funding
  - Venture capital level funding



# Possible Models

- Academic Drug Discovery and Development for Orphan Diseases
- Cell-based Therapy using induced Pluripotent Stem Cells
- Probiotic Delivery of Therapeutics

# Induced pluripotent stem (iPS) cells



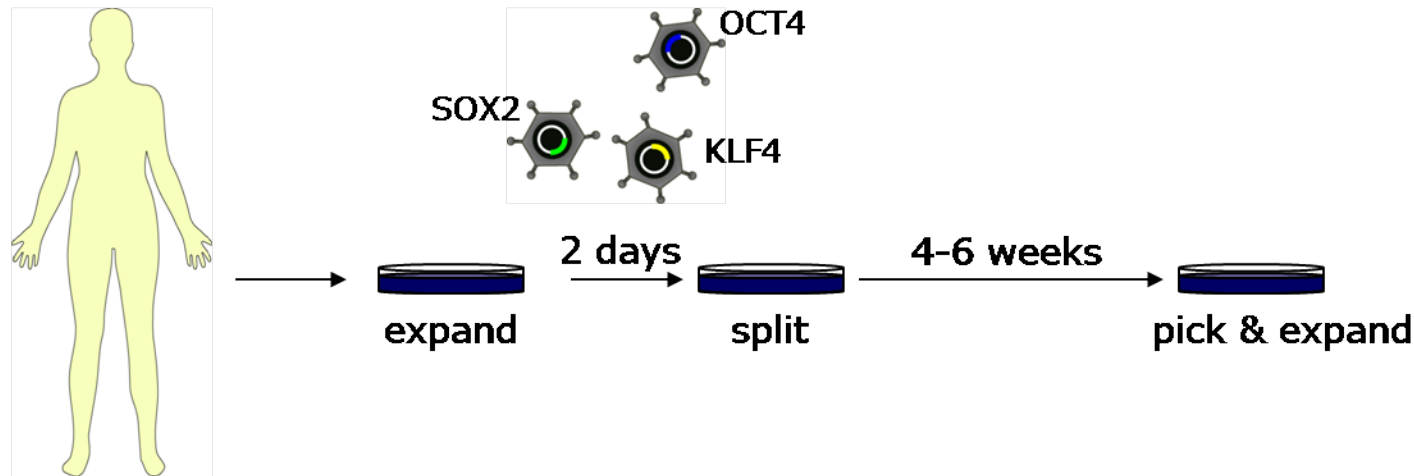
## Positive

- patient specific cells possible
  - follow disease progression
  - autologous transplantation
  - gene therapy by homologues recombination
- high availability to research community
- rapidly progressing field

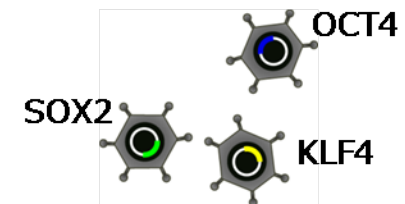
## Negative

- c-myc expression induced tumor in iPS chimeric mice
- viral integration
- limited knowledge of how iPS cells compare to ES cells

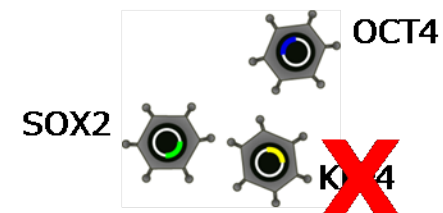
# Generation of diabetic patient specific Pluripotent Stem (DiPS) cells with genetic transfection.



**+ Valproic acid: no need for c-myc and 100 fold increase in efficiency**



**+ Valproic acid: no need for c-myc or Klf4**



# Factors for Setting Priorities

- Potential for significant health benefit
- Possible to identify specific translational steps that will lead to measurable translational advance
- Scientific teams, clinical samples, and infrastructure exist to carry out specific translational steps
- There are definitive points at which progress can be evaluated through milestones
- NIH funding critical for work to advance