

## Patient-Centered Outcomes Research Institute

- Establishes non-profit corporation (PCORI) to organize, fund CER, paid for by new trust fund
  - Overseen by Board of Directors (including Directors/designees of NIH, AHRQ)
  - Standing methodology committee (incl. Directors/designees of NIH, AHRQ) to establish research priorities; set research agenda
- Charged to identify
  - National Research Priorities
  - Evidentiary Gaps
  - New Clinical Evidence
  - Relevance; standards; economic correlates

H. R. 3590

### **Subtitle D—Patient-Centered Outcomes Research**

SEC. 6301. PATIENT-CENTERED OUTCOMES RESEARCH.

(a) IN GENERAL.—Title XI of the Social Security Act (42 U.S.C. 1301 et seq.) is amended by adding at the end the following new part:

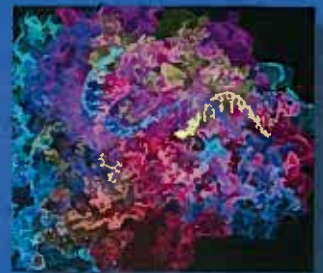
“PART D—COMPARATIVE CLINICAL EFFECTIVENESS RESEARCH

# Patient-Centered Outcomes Research Institute

- Funding
  - Direct appropriations:
    - FY 2010: \$10M
    - FY 2011: \$50M
    - FY 2012: \$150M
  - FY 2013– 2019: PCORI trust fund, \$150M plus per capita charge per enrollee (per year)
- PCORI is directed to consult with NIH on agenda, research, training, communications – NIH will continue to conduct, support CER independently

## A Bold New Paradigm: The Cures Acceleration Network (CAN)

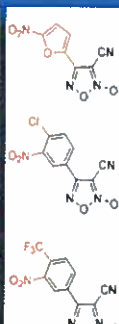
- Goal: dramatically advance development of new treatments and cures for debilitating and life-threatening diseases by reducing barriers between laboratory discoveries and clinical trials
- “promote innovation in technologies supporting the advanced research and development and production of high need cures, including through the development of medical products and behavioral therapies.”



## A Bold New Paradigm: The Cures Acceleration Network (CAN)

- Authorized budget for FY 2010: \$500M
- Situated in NIH Office of the Director
- Will provide flexible funding mechanisms
  - Grant Awards: up to \$15M per award; potential additional funding in subsequent years
  - Partnership Awards: up to \$15M, requiring matching funds (\$1 for every \$3 awarded by NIH)
  - Flexible Research Awards: DARPA-like research authority





Disease

Target ID

Assay Dev.

HTS

Probe to Lead

Pre-Clinical

FDA IND

Ph. I

Ph. II

Ph. III

FDA Review

NIH Molecular Libraries Initiative

NIH TRND

NIH RAID

Pharma, Biotech, NIH Clinical Center, CTSAs

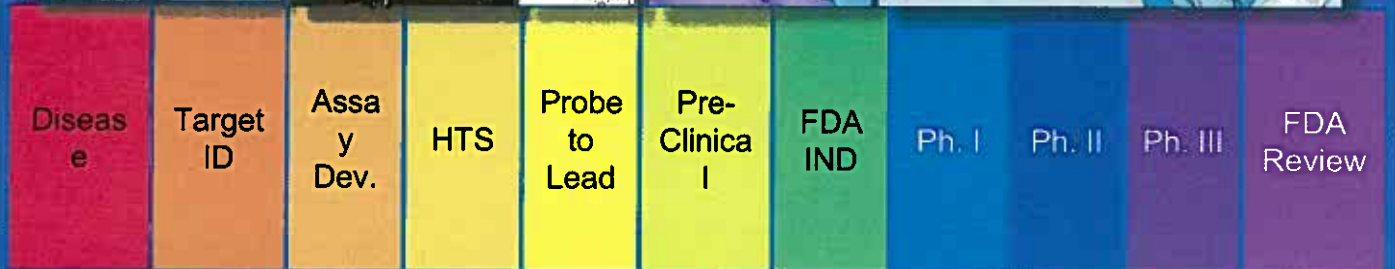
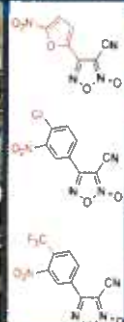
New NIH FDA Partnerships

## New NIH-FDA Partnership

- NIH FDA Joint Leadership Council
  - Improve translational research
  - Make our science “regulatory review ready”
  - Speed development of new medical products
- Joint Regulatory Science Initiative
  - 59 letters of intent
  - \$6.75 M
- Public Consultation: June 2, 2010



# Cures Acceleration Network



NIH Molecular Libraries Initiative

NIH TRND

NIH RAID

Pharma, Biotech, NIH Clinical Center, CTSAs

New NIH FDA Partnerships

# A good cure requires a good diagnosis



## HIGH NEED CURE-

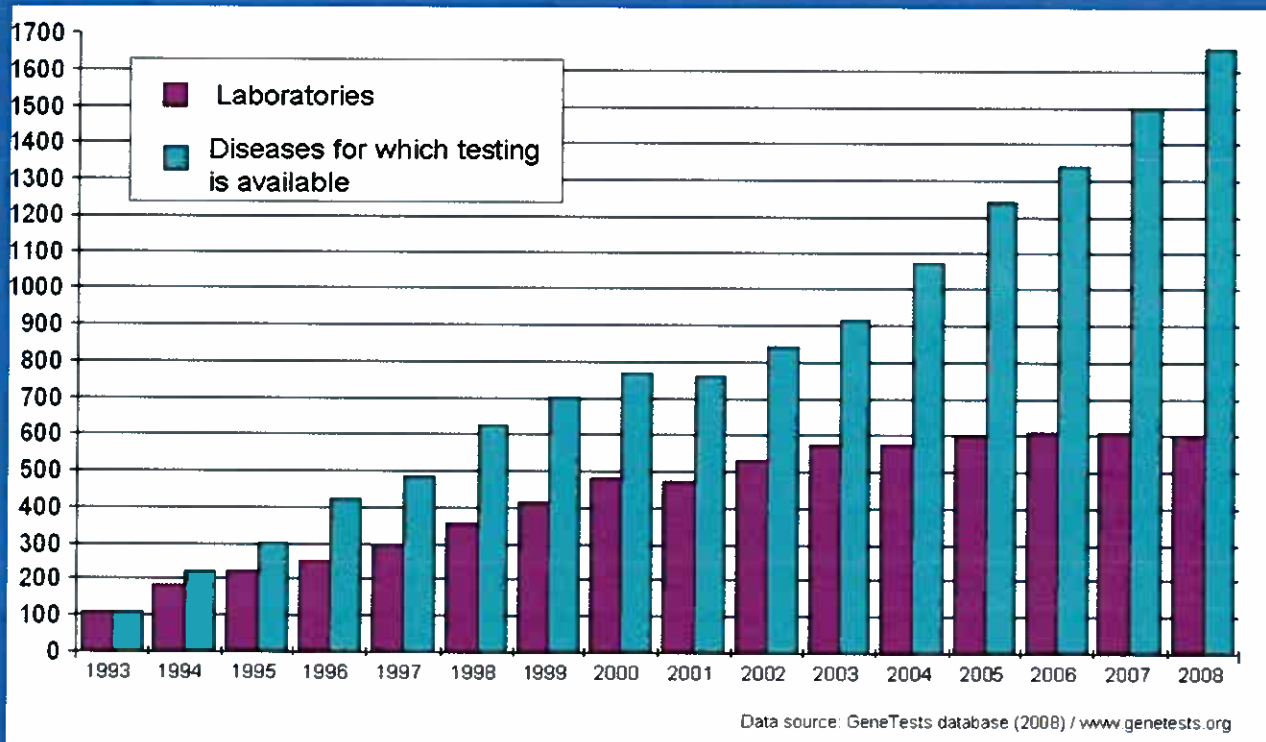
The term 'high need cure' means a drug ..., biological product..., or device... that, in the determination of the Director of NIH--

(A) is a priority to diagnose, mitigate, prevent, or treat harm from any disease or condition; and

(B) for which the incentives of the commercial market are unlikely to result in its adequate or timely development.

# Genetic testing has changed dramatically...

- Increase in number



## Genetic testing has changed dramatically...

- Increase in number
- Increase in complexity

Detection of single mutations  
or massive chromosomal  
alterations



Detection of millions of SNPs,  
whole genome sequencing,  
expression analysis, CGH, etc.

