Exceptional Opportunities for Biomedical Research

Francis S. Collins, M.D., Ph.D.
Director, National Institutes of Health

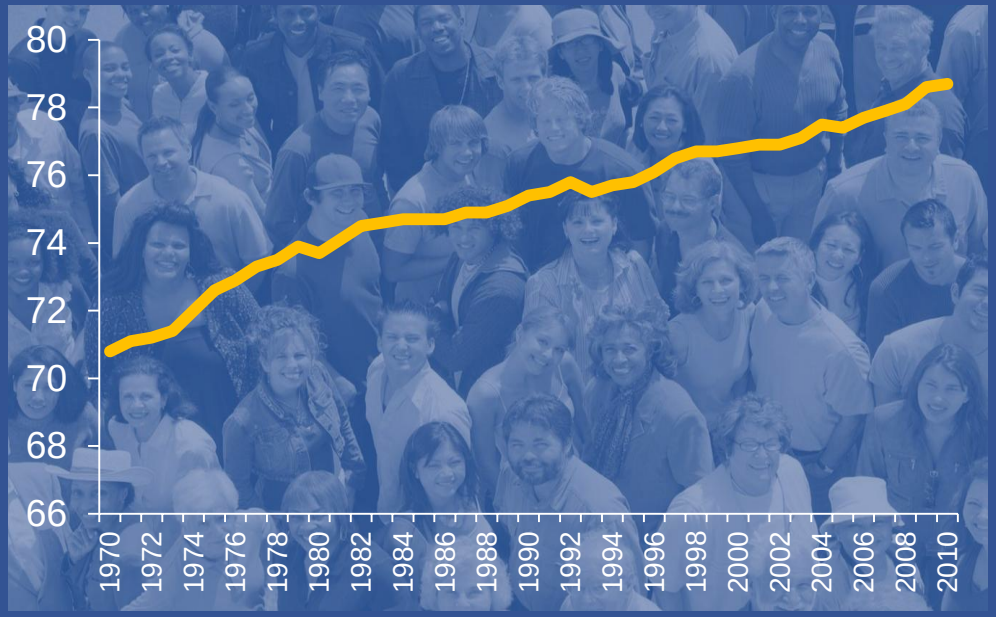
Council of Councils
June 5, 2012

NIH: Steward of Medical and Behavioral Research for the Nation

"Science in pursuit of fundamental knowledge about the nature and behavior of living systems… and the application of that knowledge to extend healthy life and reduce the burdens of illness and disability."

NIH's Impact on U.S. Health and Medicine

U.S. Life Expectancy



NIH Accomplishments

Reduction in deaths from:

- Heart disease
- Stroke
- HIV/AIDS

Increased survival rates for:

- Breast cancer
- Cervical cancer
- Colon cancer

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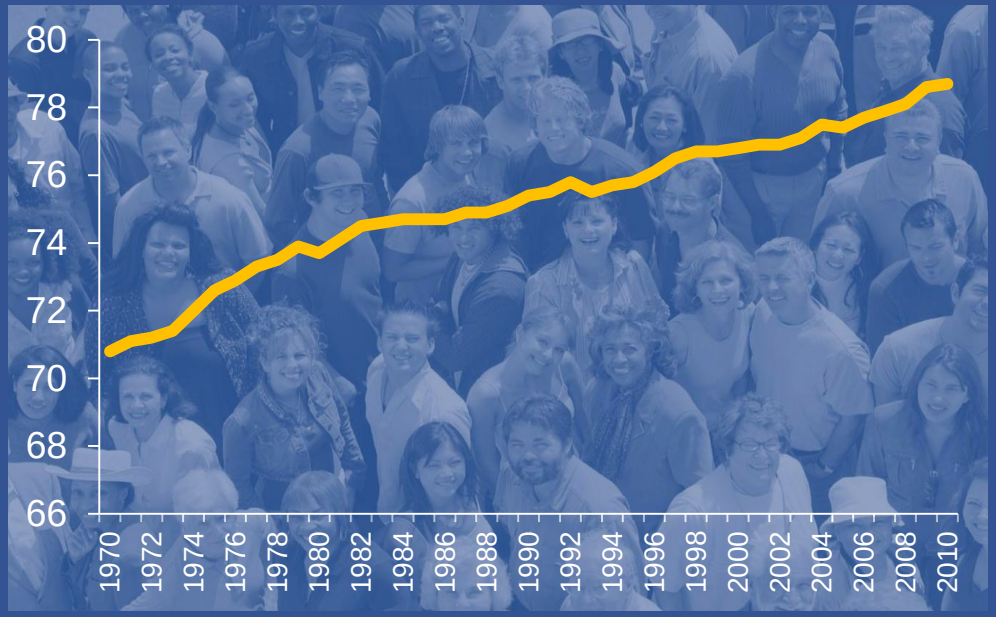


Life expectancy gains worth ~$3.2 trillion annually

NIH Accomplishments

Cardiovascular disease death rates have fallen > 60% in the last half-century

HIV therapies enable people in their 20s to live to age 70+

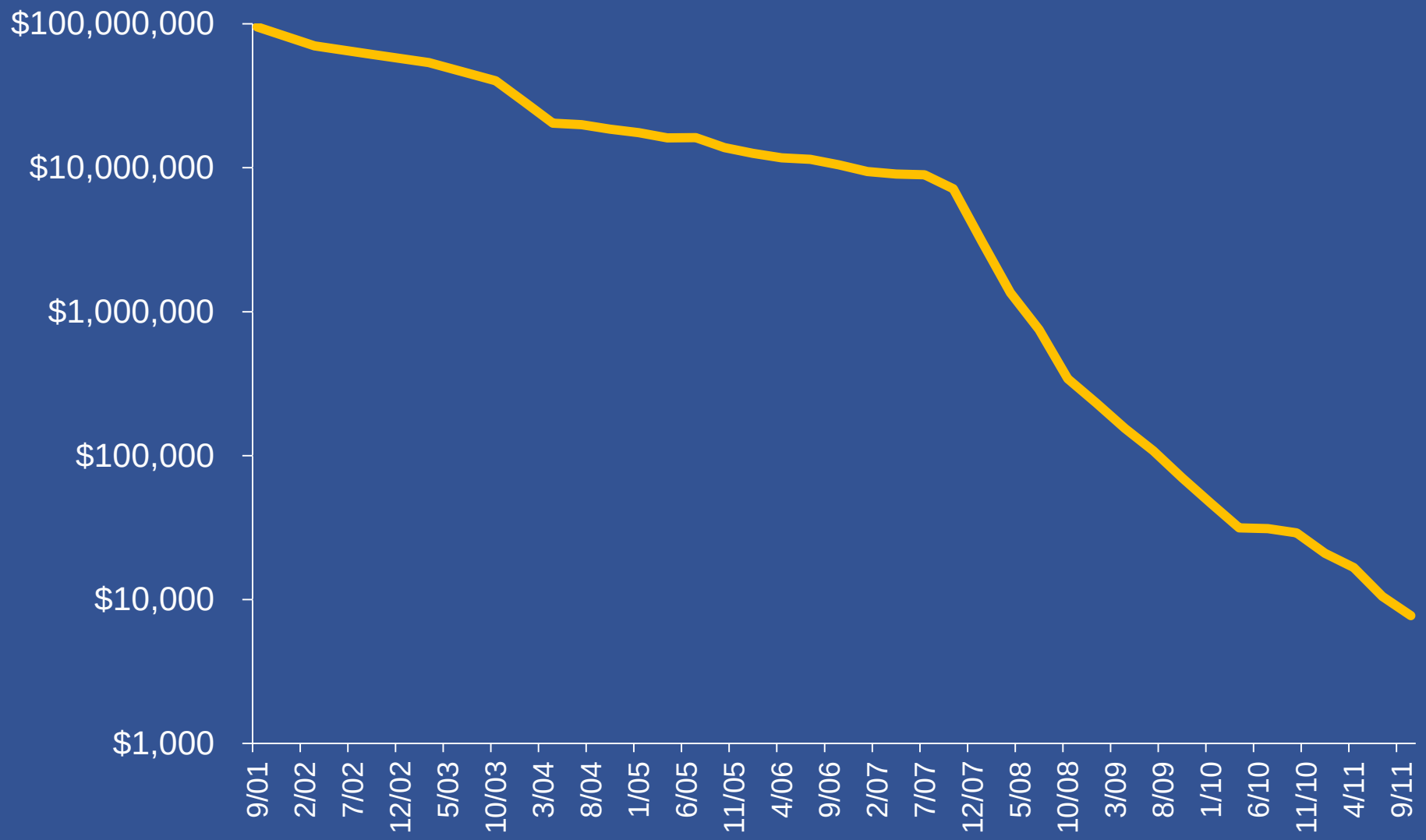
Cancer death rates falling ~1% per year; each 1% drop saves ~$500 billion

NIH Investments in Innovation

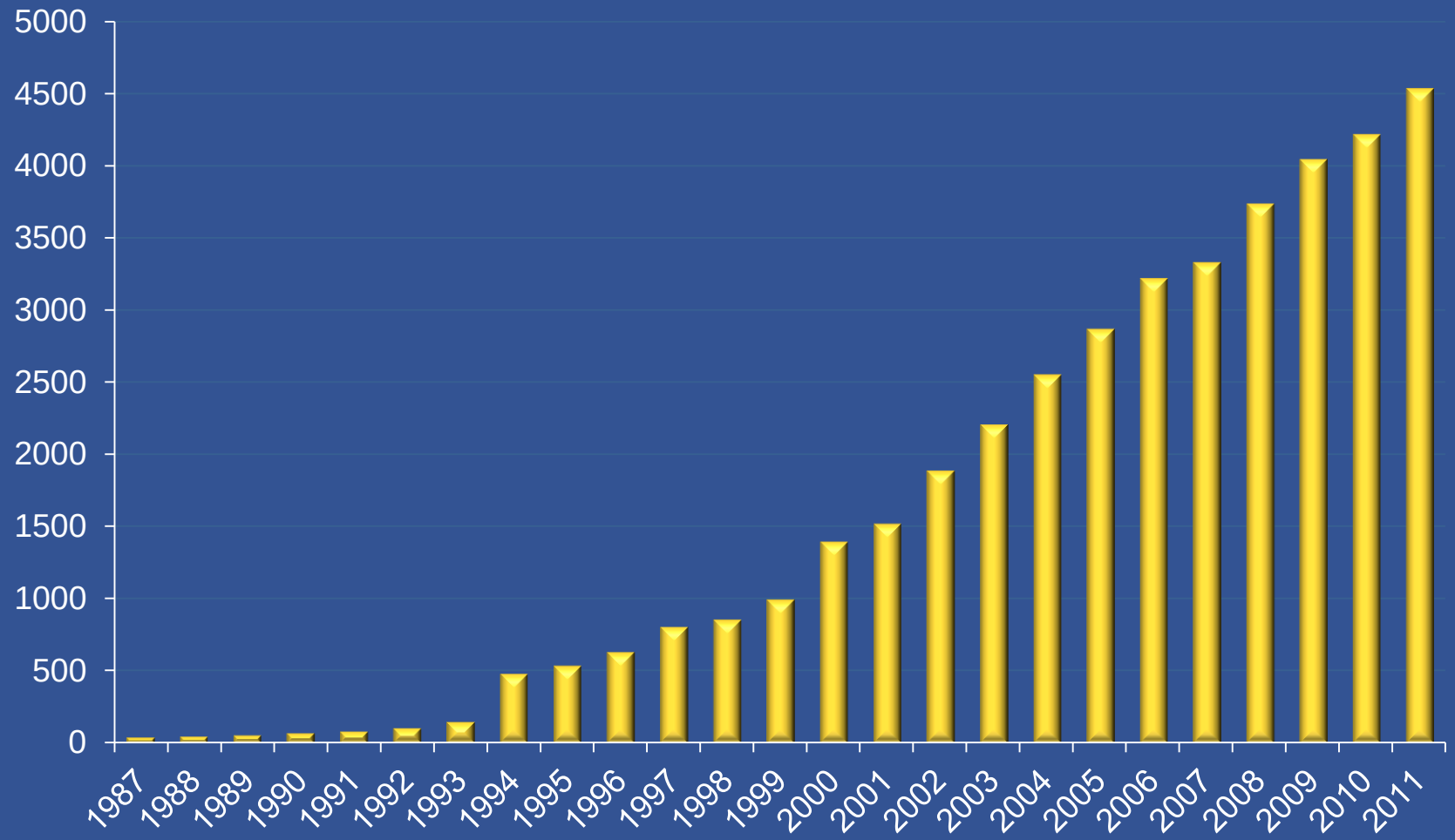
- Technology
- Translation
- Talent
- Taxpayer Return on Investment

Cost of Sequencing a Human Genome

2001–2011

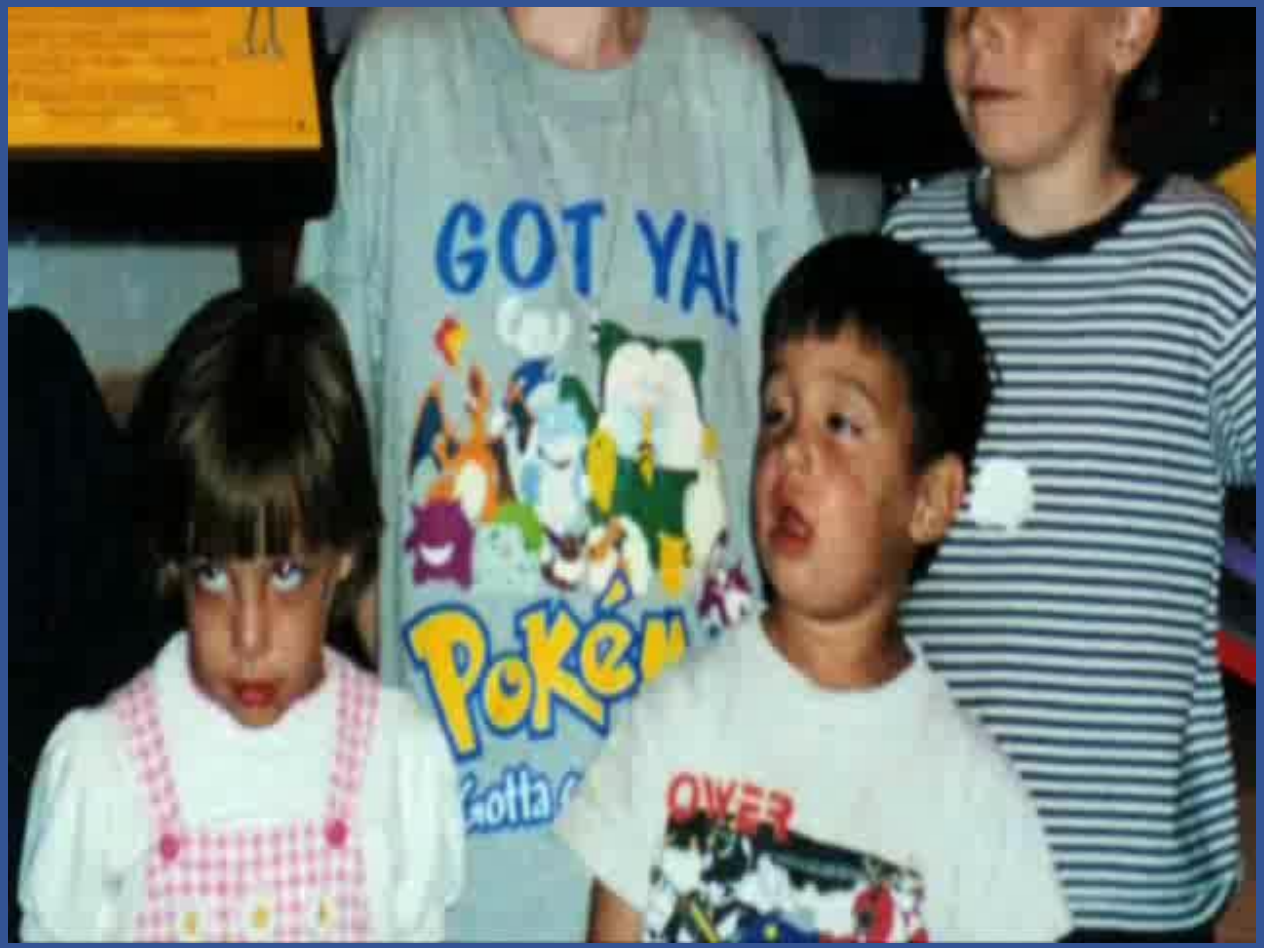


Disorders with Known Molecular Basis



Source: Online *Mendelian Inheritance in Man*, Morbid Anatomy of the Human Genome

Success Story: Noah and Alexis Beery



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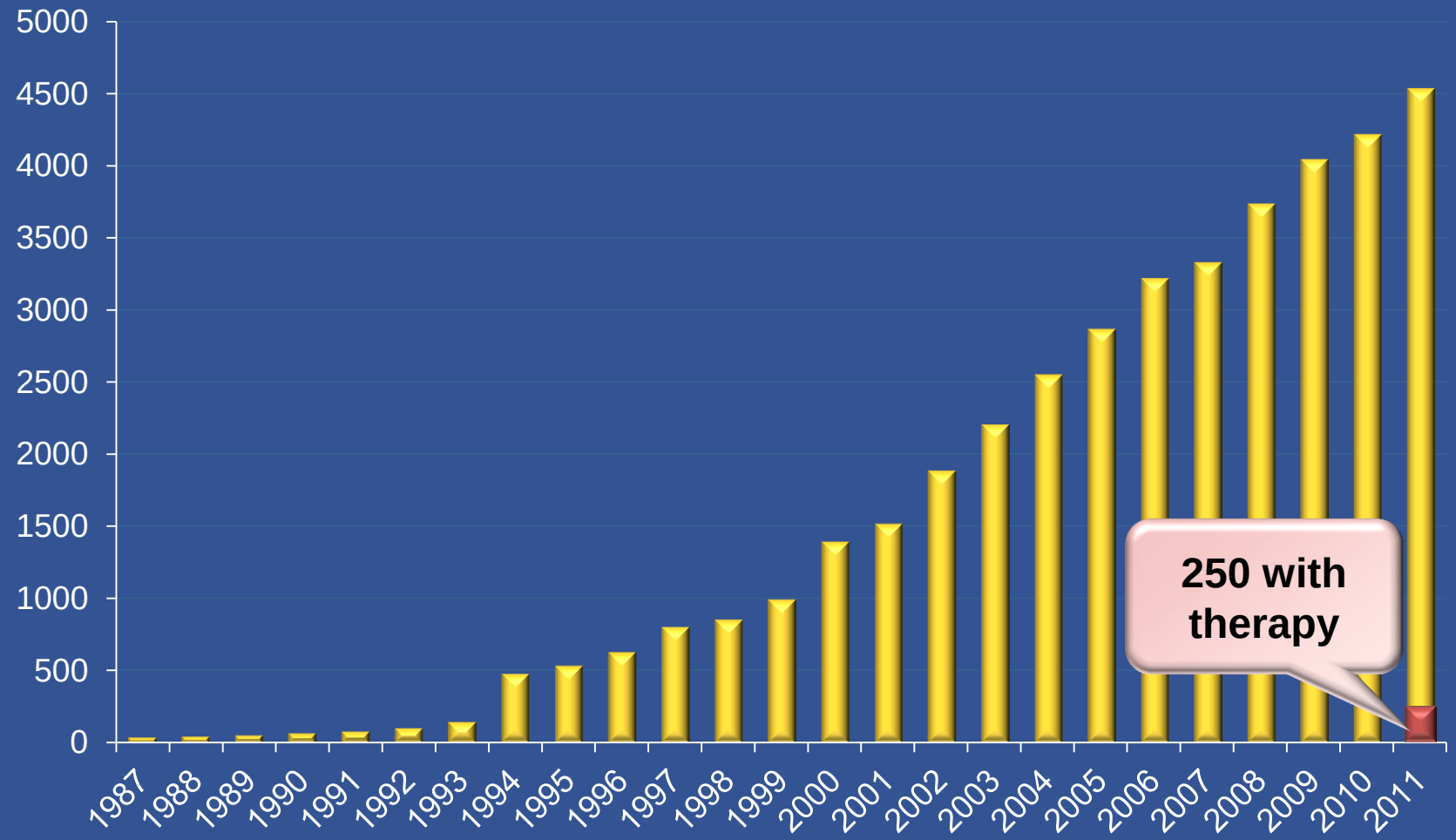

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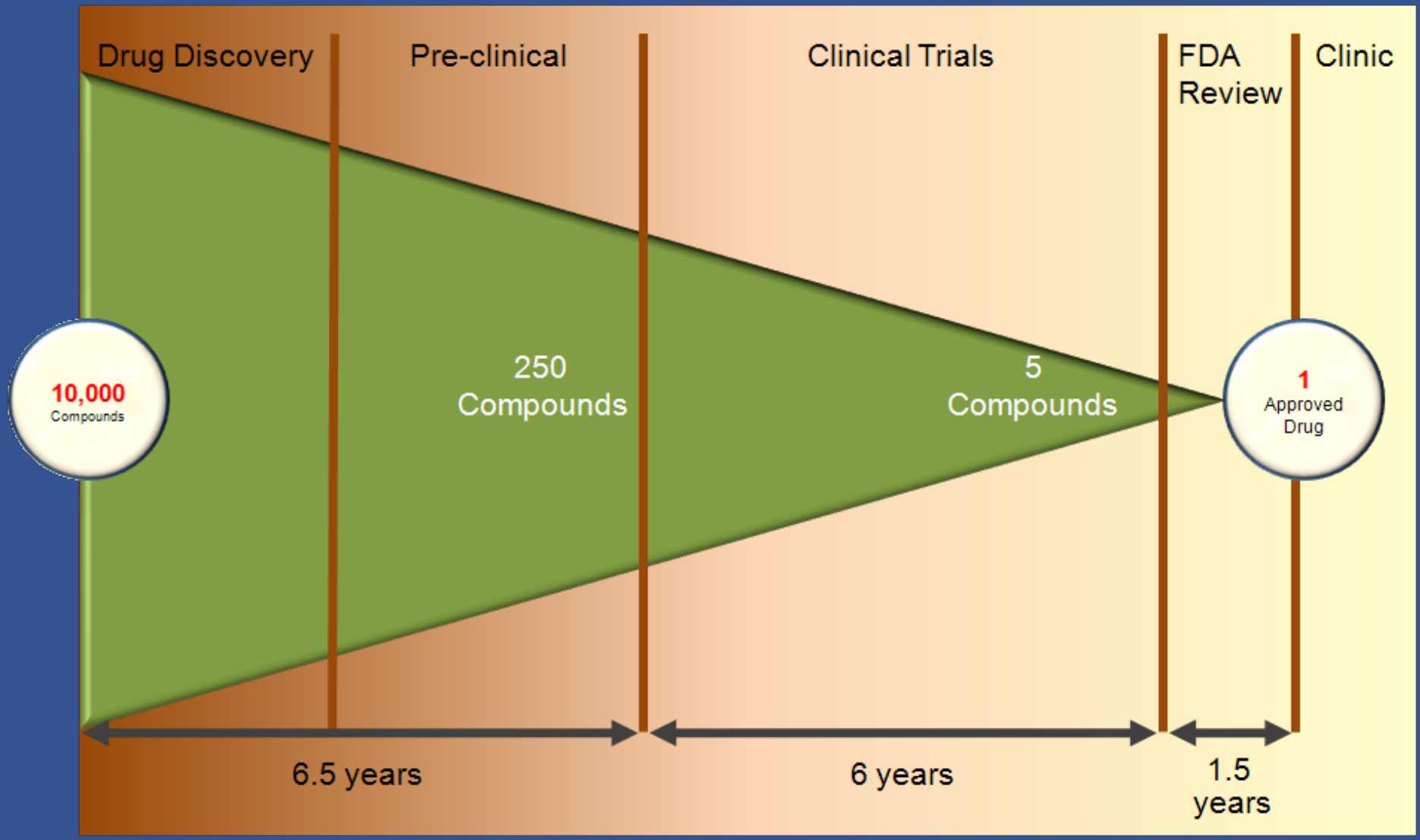
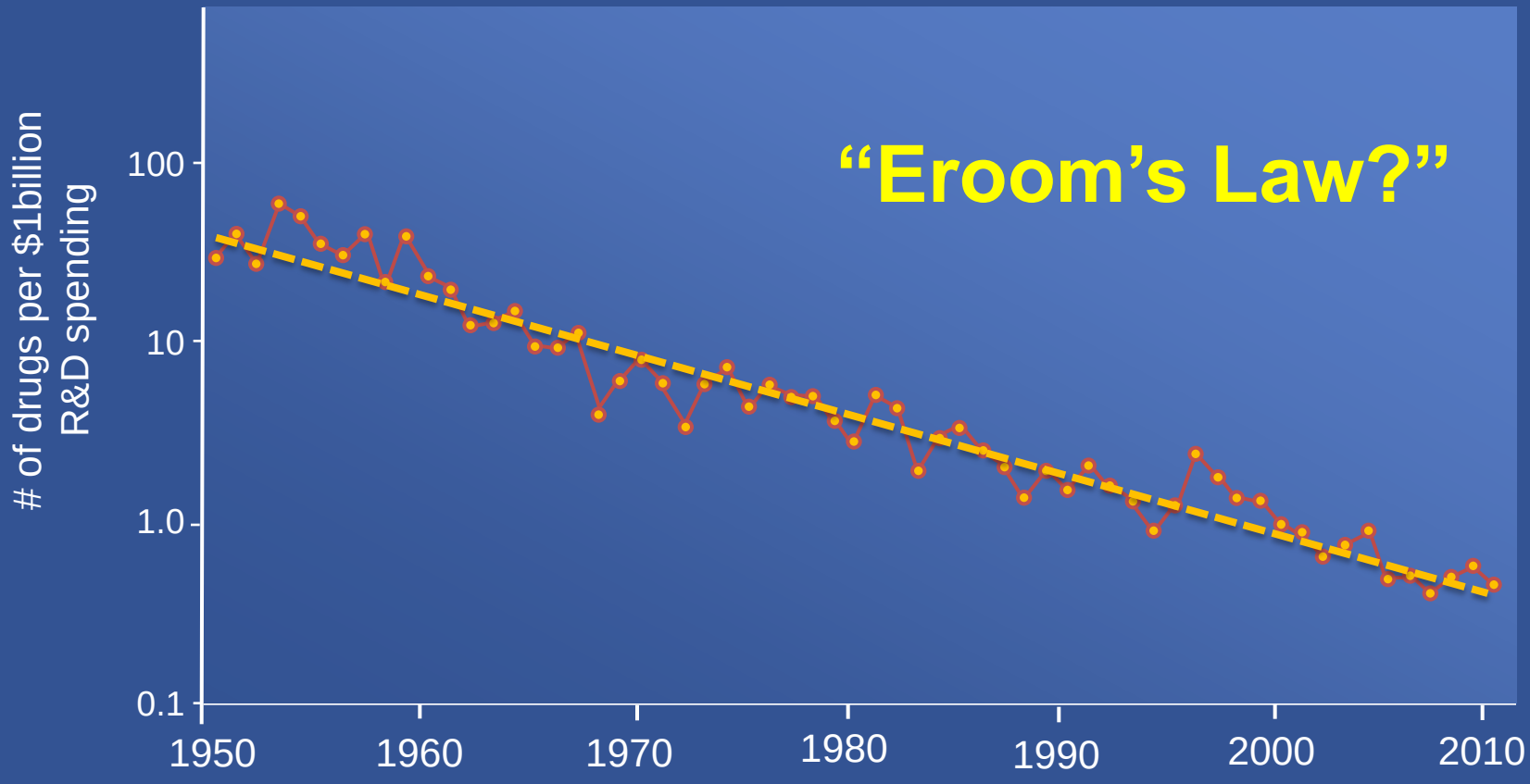
Advancing Translational Sciences



A Frustrating Decline in the Development of New Therapeutics

Overall trend in productivity (adjusted for inflation)



Source: Scannell JW et al. *Nat Rev Drug Discov.* ***11***, 191 201 (2012).

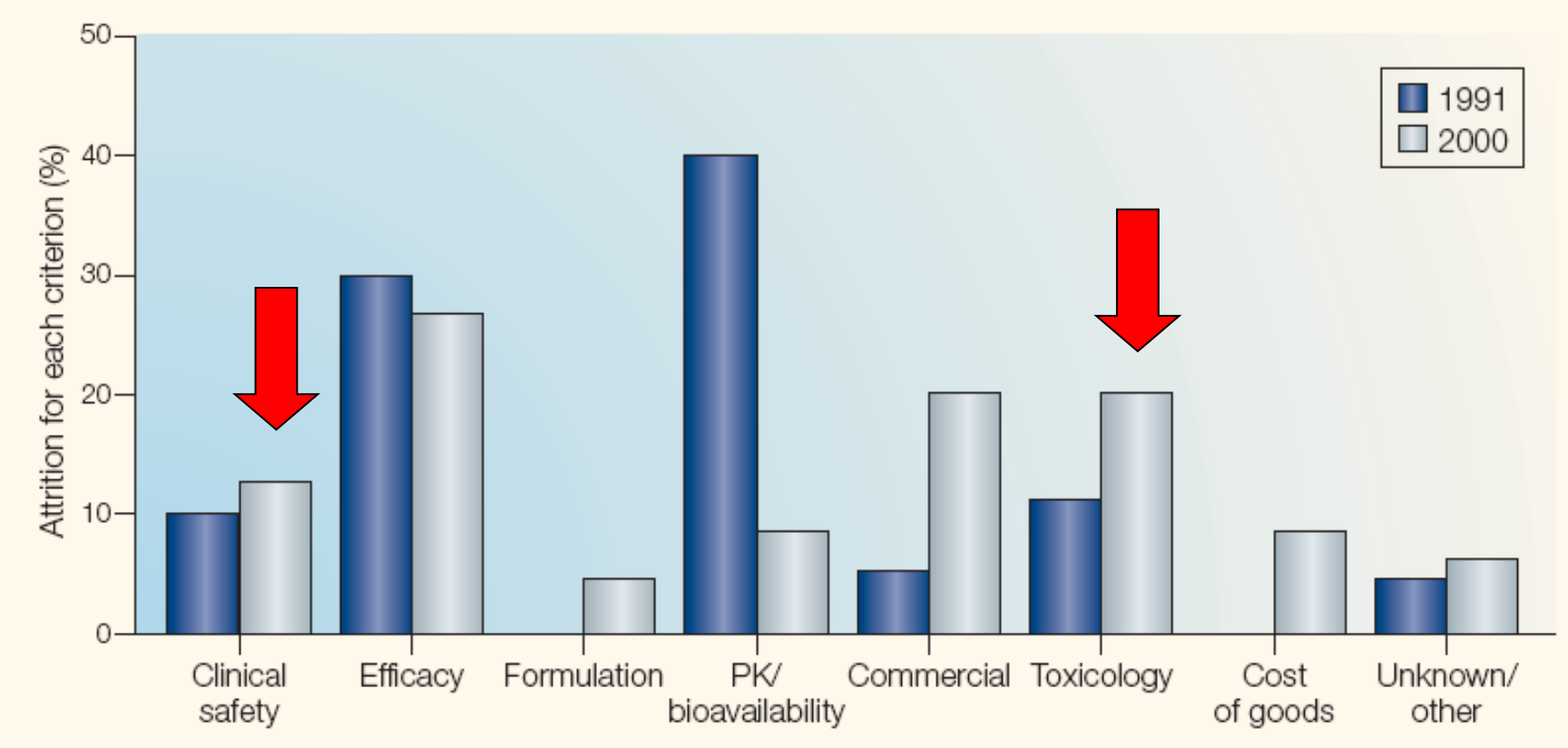
National Center for Advancing Translational Sciences (NCATS)

Mission:

To catalyze the generation of innovative methods and technologies that will enhance the development, testing, and implementation of diagnostics and therapeutics across a wide range of human diseases and conditions.

http://ncats.nih.gov/

Toxicity is the Most Common Reason for Drug Development Failure



Preclinical (21%) + Clinical (12%) Tox = 33% of all failures

Kola and Landis, *Nature Reviews Drug Discovery* 3, 711-716, 2004

Drug Rescue and Repurposing

NIH, companies aim to teach old drugs new tricks

May 3, 2012 By The Associated Press LAURAN NEERGAARD (AP Medical Writer)

(AP) -- Three pharmaceutical giants are unlocking th
scientists can reinvent some of their old drugs.

Pfizer, AstraZeneca and Eli Lilly & Co. entered a un
of Health on Thursday that both sides hope will spe
by dusting off two dozen old drugs that failed to trea

THE WALL STREET JOURNAL.

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U.S. to Seek New Uses for Abandoned Drugs

BY THOMAS M. BURTON

The U.S. government said Thursday it will work with large pharmaceutical companies to try to find new uses for once-promising drugs that have been cast aside by the industry.

The National Institutes of Health initially will work with Pfizer Inc., Eli Lilly & Co. and AstraZeneca PLC to match abandoned drugs with researchers from universities, hospitals and the NIH.

Researchers at the NIH's new National Center for Advancing Translational Sciences generally will focus on drugs known to work on a specific gene but that somehow failed in initial testing by

Drug Rescue and Repurposing:
Novel NIH-Industry Collaboration

- Pfizer, AstraZeneca, Eli Lilly will supply dozens of compounds that:
  - Were tested in human clinical trials
  - Have acceptable safety profiles but were de-prioritized for lack of efficacy or change in business directions
- NIH will match industry compounds with academic, biotech, non-profit researchers to find new uses
- Features template agreements to:
  - Reduce time, cost, effort
  - Provide roadmap for handling intellectual property

NCATS Catalyzing Innovation

AstraZeneca

Pfizer

Lilly

FDA-NIH Tobacco Control Regulatory Science Program

- Family Smoking Prevention and Tobacco Control Act (2009) gives FDA authority to regulate manufacture, distribution, marketing of tobacco products to protect public health
- NIH-FDA Leadership Tobacco Regulatory Science WG
  - Co-chairs: Drs. Tom Insel, NIMH/NCATS; Bob Croyle, NCI
- Following appointment of Associate Director for Disease Prevention, NIH Office of Disease Prevention will:
  - Coordinate research activities on behalf of NIH
  - Review and/or prepare FOAs
  - Oversee tobacco control research funding
  - Conduct portfolio analysis and mapping to 56 FDA tobacco control research priorities
  - Expand collaborations and partnerships

http://cancercontrol.cancer.gov/nih-fda/

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NIH: Investing in People and Ideas

- Ruth L. Kirschstein National Research Service Awards
- NIH Director's Early Independence Awards
- NIH-Lasker Clinical Research Scholars Program
- New Innovator Award
- Transformative R01
- NIH Director's Pioneer Award

Encouraging a More Balanced Workforce

- African Americans, Hispanics, and Native Americans:
  - Represent **31%** of U.S. college age population
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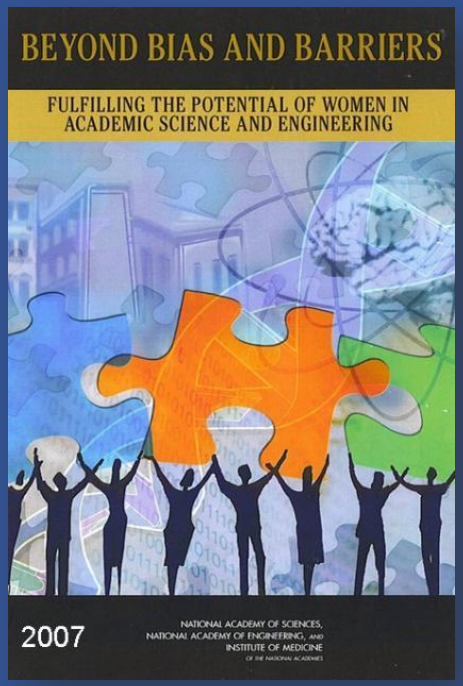
- Racial – and gender – barriers persist at later stages of career development



NEWS & ANALYSIS

BIOMEDICAL RESEARCH FUNDING

NIH Uncovers Racial Disparity in Grant Awards

STUDY AT A GLANCE

83,188	R01 applications from Ph.D.s analyzed
40,069	Unique Ph.D. investigators
1149	R01 applications from black Ph.D.s
337	Expected awards to black applicants if same success chance as whites
185	Actual awards to black applicants

Science
VOL 333 19 AUGUST 2011

Race, Ethnicity, and NIH Research Awards

Donna K. Ginther,[1]* Walter T. Schaffer,[2] Joshua Schnell,[3] Beth Masimore,[3] Faye Liu,[3] Laurel L. Haak,[3] Raynard Kington[2]†

Encouraging a More Balanced Workforce

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- Racial – and gender – barriers persist at later stages of career development
- Efforts to increase research workforce diversity
  - NIH Working Group on Women in Biomedical Careers
  - Systematic effort to remove bias from peer review process

POLICYFORUM

SOCIOLOGY

Weaving a Richer Tapestry in Biomedical Science

NIH leadership discusses the need for renewed efforts to increase diversity in the U.S. biomedical research workforce.

Lawrence A. Tabak* and Francis S. Collins*

As much as the U.S. scientific community may wish to view itself as a single garment of many diverse and colorful threads, an unflinching consideration ...ls us that our nation's bio- ...rkforce remains nowhere ...uld be. An analysis, per- ... of researchers primar- ...e National Institutes of ...ublished in this issue of ...at from 2000 to 2006, ...licants were significantly ...e NIH research funding ...plicants. The gap in suc- ...l to 10 percentage points, ...ng for education, coun- ...g, employer characteris- ...rch awards, and publica- tion record (2). Their analysis also showed a gap of 4.2 percentage points for Asians; however, the differences between Asian and white

Encouraging Students to Pursue Science, Technology, Engineering, and Math Careers

- Evaluation
  - Formal vs. informal science education efforts
  - Best practices
- Do more with less
  - Trans-NIH STEM (Science Education Research Group)
- Strategic Planning
  - Trans-Agency STEM
  - NIH STEM
- Proposed Council of Councils STEM Education Working Group

"A teacher affects eternity, he can never tell where his influence stops."

~ Henry B. Adams

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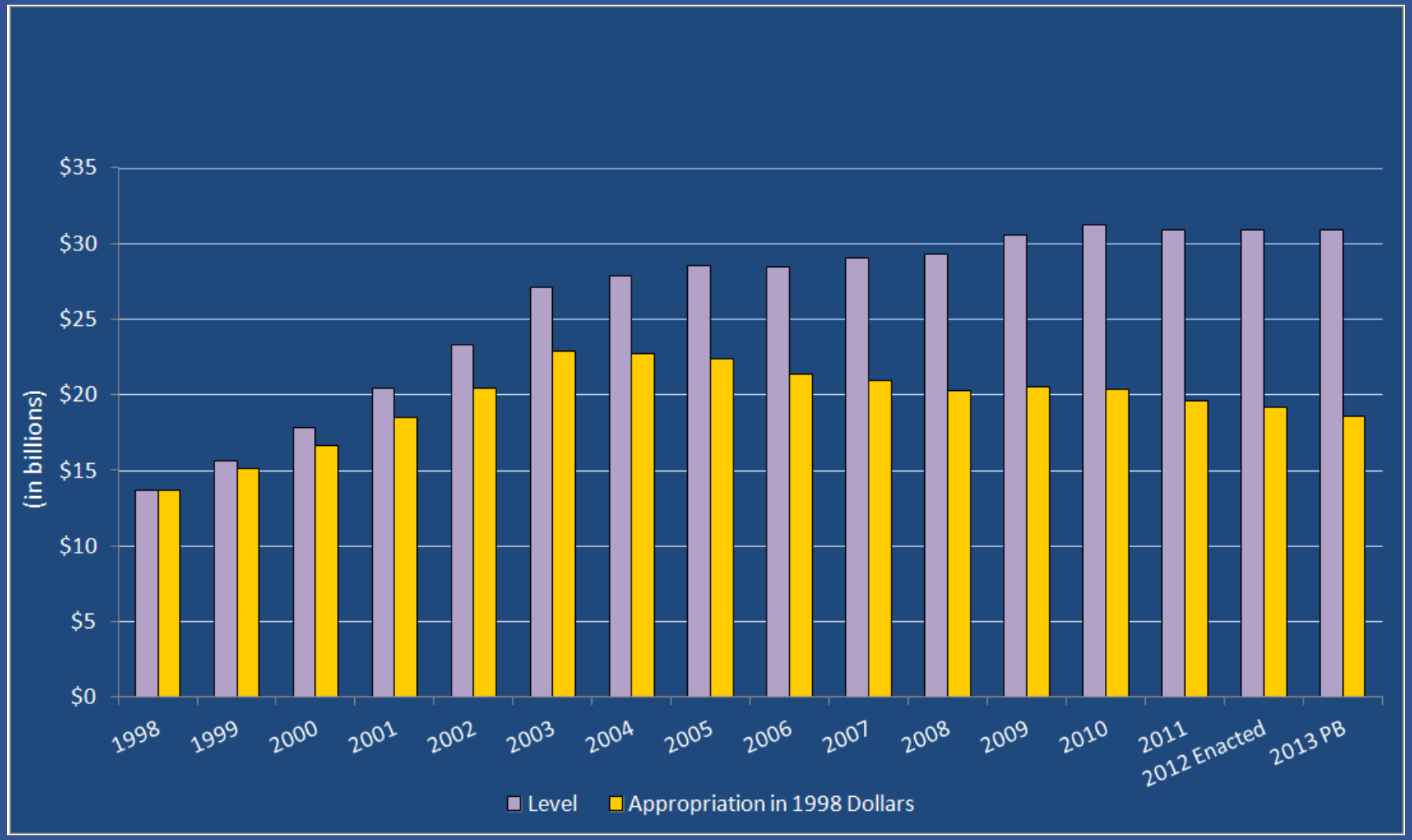
The Effects of Inflationary Growth on Purchasing Power



Impact of NIH-Supported Research On U.S. Economy

- In 2010, NIH research supported 488,000 jobs at 3000 institutions, small businesses nationwide
- In 2010, NIH funding generated $68 billion in new economic activity—double taxpayers' investment
- NIH serves as foundation for entire U.S. medical innovation sector that:
  - Employs 1 million U.S. citizens
  - Generates $84 billion in wages, salaries
  - Exports $90 billion in goods, services

Source: An Economic Engine: NIH Research, Employment and the Future of the Medical Innovation Sector, United Medical Research, May 2011

"If we're going to create jobs now and in the future, we're going to have to out-build and out-educate and out-innovate every other country on Earth."

President Obama
Signing of America Invents Act
Thomas Jefferson High School
September 16, 2011




NIH…

Turning Discovery Into Health