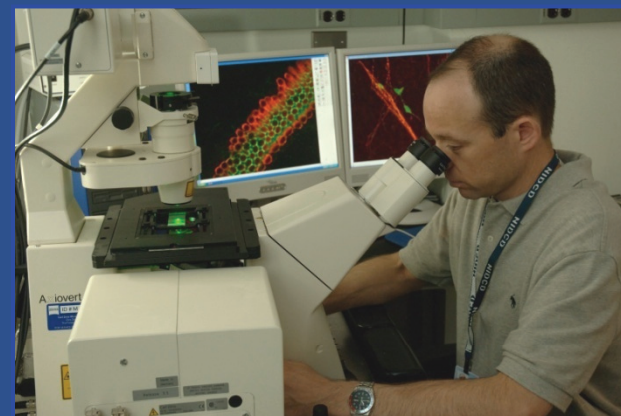


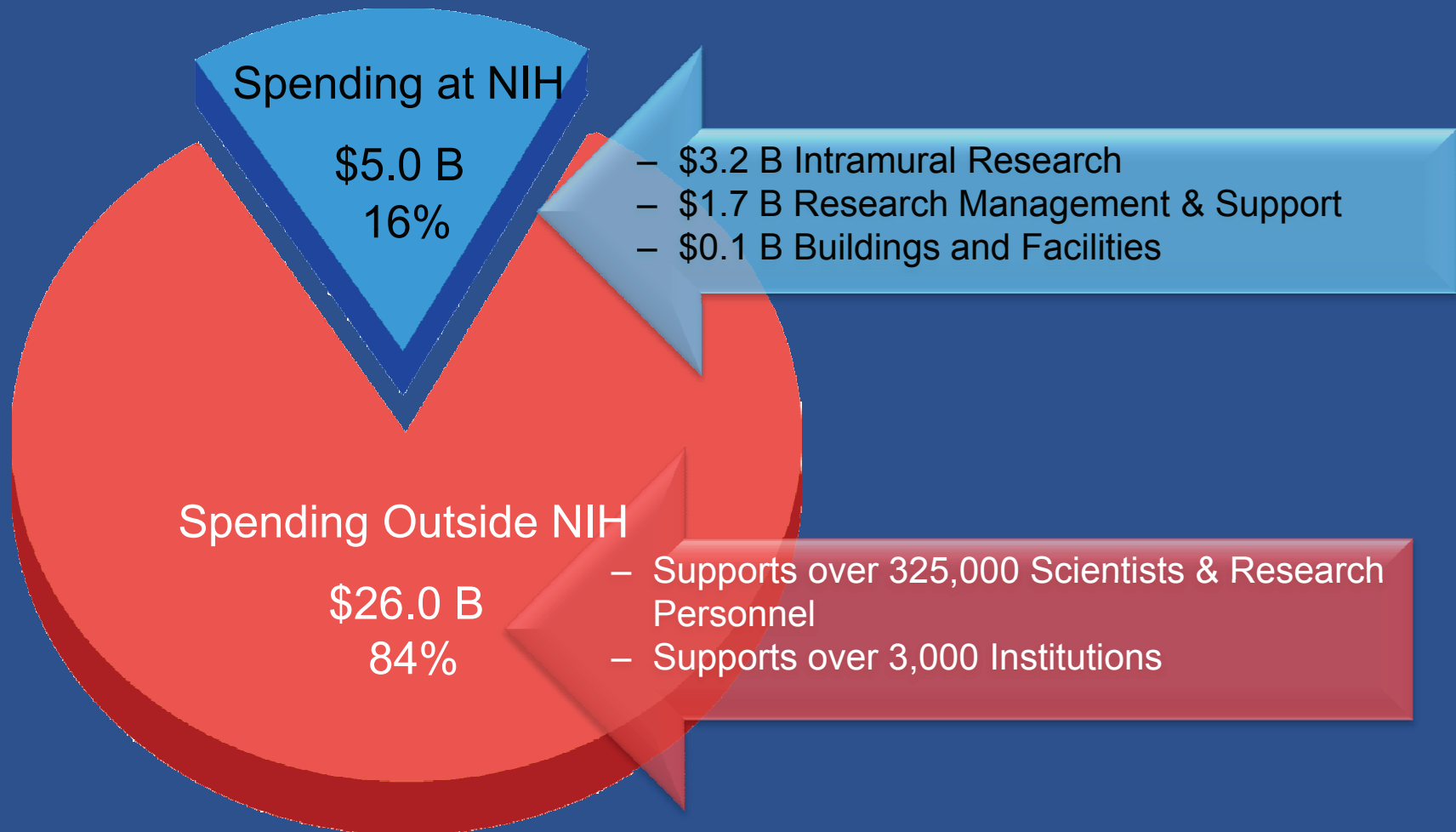
NIH: Steward of Medical and Behavioral Research for the Nation



“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.”



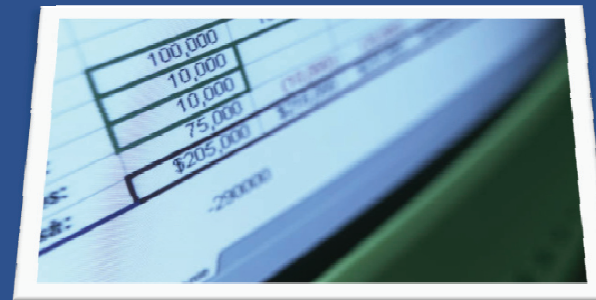
NIH Extramural & Intramural Funding FY 2010 Final Budget: \$30.988 Billion*



* Includes \$150 million from the Special type 1 Diabetes appropriation.

Recovery Act Appropriated \$10 Billion Directly to NIH

- 13,000 grant awards
- 1,885 new investigators
- 28 institutions are first-time NIH awardees, 12 of which are small businesses
- Estimated job creation/retention: 50,000 over 2 years
- Every \$1 of NIH grant funding returns \$2.21 in goods and services in one year



The President visits NIH on Sept. 30, 2009

- *to highlight ARRA awards*
- *to reiterate his strong support for science*



Opportunities for Research and NIH

Francis S. Collins

The mission of the National Institutes of Health (NIH) is 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 to reduce the burdens of illness and disability. The power of the molecular approach to health and disease has steadily gained momentum over the past several decades and is now poised to catalyze a revolution in medicine. The foundation of success in biomedical research has always been, and no doubt will continue to be, the creative insights of individual investigators. But increasingly those investigators are working in teams, accelerated by interdisciplinary approaches and empowered by open access to tools, databases, and technologies, so a careful balance is needed between investigator-initiated projects and large-scale community resource programs. For both individual and large-scale efforts, it is appropriate to identify areas of particular promise. Here are five such areas that are ripe for major advances that could reap substantial downstream benefits.

High-Throughput Technologies

In the past, most biomedical basic science projects required investigators to limit their scope to a single aspect of cell biology or physiology. The revolution now sweeping the field is the ability to be comprehensive—for example, to define all of the genes of the human or a model organism, all of the human proteins and their structures, all of the common variations in the genome, all of the major pathways for signal transduction in the cell, all of the patterns of gene expression in



sive information about the genetic underpinnings of 20 major tumor types. This information will likely force a complete revision of diagnostic categories in cancer and will usher in an era where abnormal pathways in specific tumors will be matched with the known targets of existing therapeutics. Another example is the opportunity to understand how interactions between ourselves and the microbes that live on us and in us (the “microbiome”) can influence health and disease (2).

Translational Medicine

Critics have complained in the past that NIH is too slow to translate basic discoveries into new diagnostic and treatment advances in the clinic. Some of that criticism may have been deserved, but often the pathway from molecular insight to therapeutic benefit was just not

The promise of fundamental advances in diagnosis, prevention, and treatment of disease has never been greater.

1 JANUARY 2010 VOL 327 SCIENCE

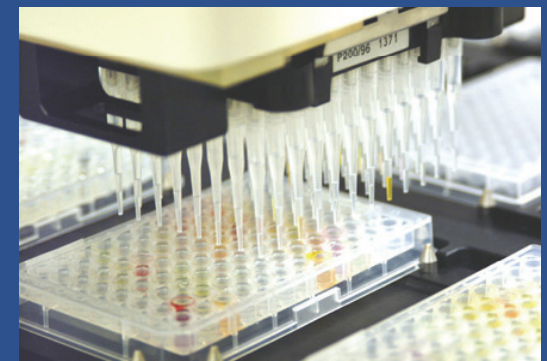
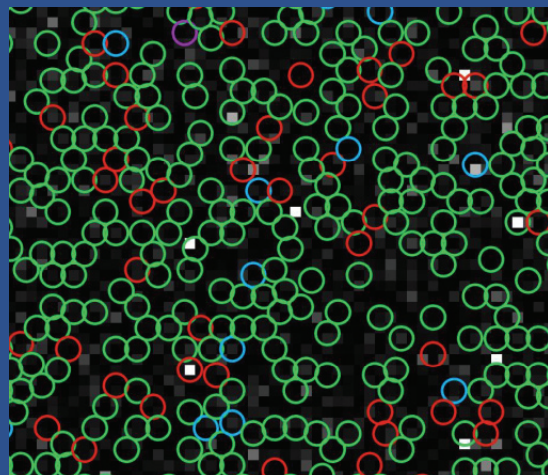
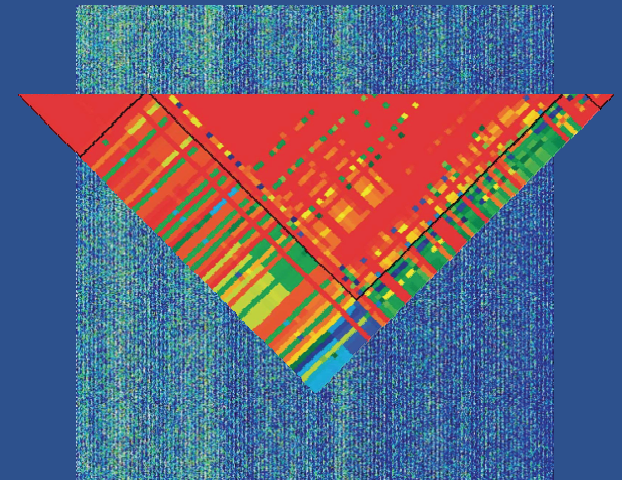
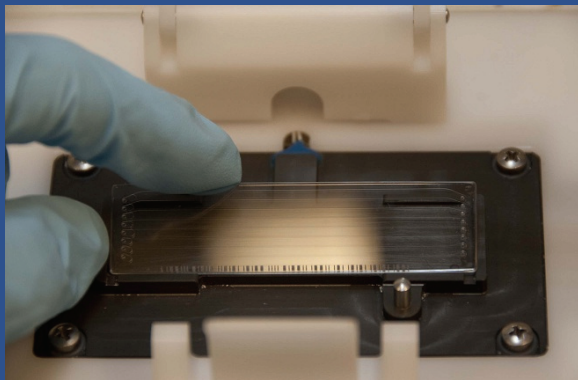
Published by AAAS

bring them to clinical trials and U.S. Food and Drug Administration (FDA) approval.

As one example, the NIH Therapeutics for Rare and Neglected Diseases (TRND) (3) program will allow certain promising compounds to be taken through the preclinical phase by NIH, in an open environment where the world’s experts on the disease can be involved. Furthermore, as information about common diseases increases, many are being resolved into distinct molecular subsets, and so the TRND model will be even more widely applicable.

The first human protocol (for spinal cord injury) involving human embryonic stem cells (hESCs) was approved by the FDA in 2009, and the opening up of federal support for hESC research will bring many investigators into this field. The capability of transforming human skin fibroblasts and other cells into induced pluripotent stem

Opportunity 1: Using high throughput technologies to understand fundamental biology, and to uncover the causes of specific diseases



- **NextGen DNA sequencing and beyond**
- **Nanotechnology**
- **Small molecule screening**
- **New imaging modalities**
- **Emphasis on comprehensive approaches**
 - *All of the genes, all of the proteins, all of the common variations, all of the pathways for signal transduction, all of the patterns of gene expression, all of the steps in early development, all of the components of the immune system...*
- **Computational biology is critical**
- **Examples that are ripe for expanded effort**
 - **Cancer**
 - **Autism**
 - **Microbiome**
 - **Many more....**

Opportunity #2: Translating basic science discoveries into new and better treatments



- **Stage is set for NIH to play a greatly expanded role in translation**
 - New discoveries about the fundamental basis of disease
 - Resources empowering academic investigators to develop lead compounds and “de-risk” projects
 - Opportunity for public-private partnerships
- **Stem cells (including hESC and iPSC)**
 - Explosion of new information likely with new NIH policy – 51 cell lines now approved for federal funding
 - Therapeutic uses still uncertain but urgent to pursue
- **Small molecules**
 - Roadmap provides high throughput screening (HTS) capabilities that now match that of many pharmaceutical companies
 - **More on this theme later in the talk**

Opportunity #3: Putting science to work for the benefit of health care

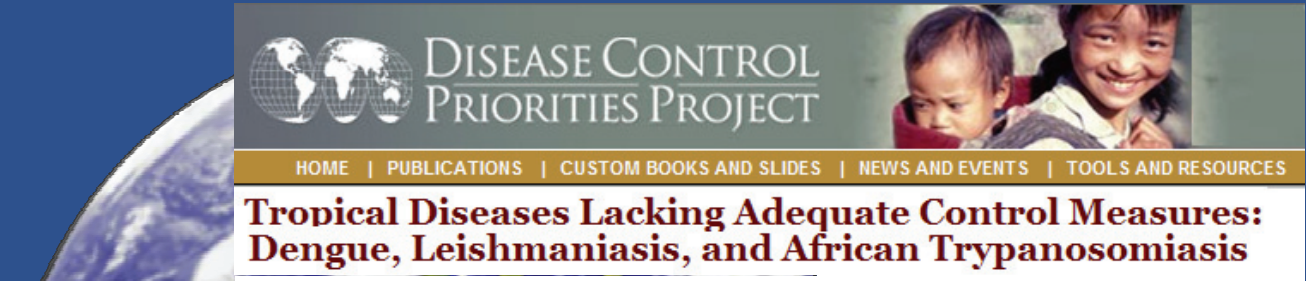


- **Comparative effectiveness research**
- **Prevention and personalized medicine**
 - Behavioral research
- **Health disparities research**
- **Pharmacogenomics**
- **Large scale prospective studies**
 - National Children's Study
 - Study of adult onset diseases?
- **Health IT**
- **Health research economics**
 - Going beyond clinical trials to studying health care delivery in the real world?
 - What payment incentives actually work to reduce costs and improve outcomes?

Opportunity #4: Encouraging a greater focus on global health

Depression

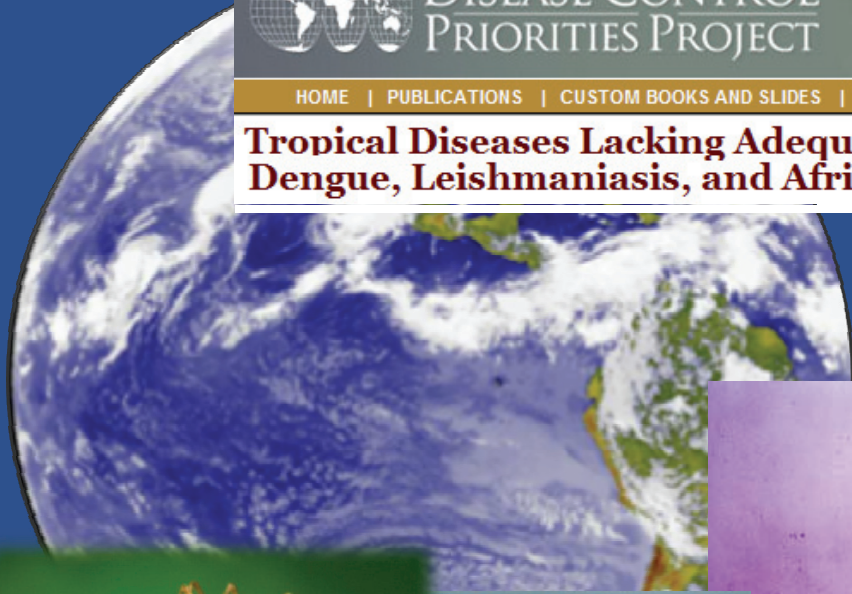
Leading cause
of disability
worldwide...



DISEASE CONTROL
PRIORITIES PROJECT

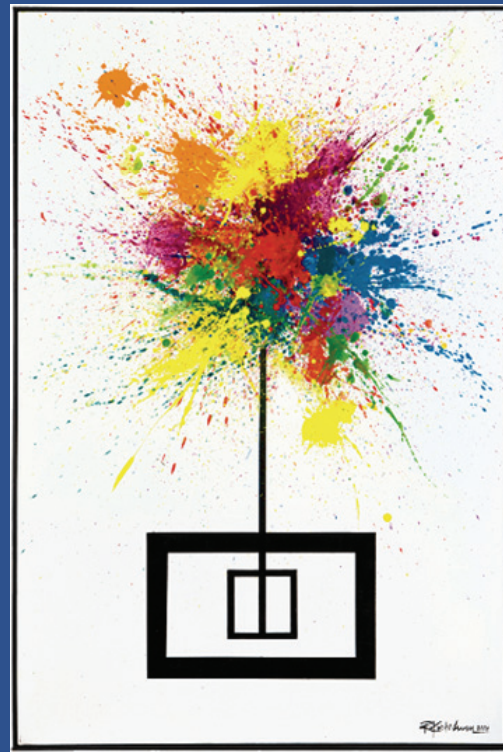
HOME | PUBLICATIONS | CUSTOM BOOKS AND SLIDES | NEWS AND EVENTS | TOOLS AND RESOURCES

Tropical Diseases Lacking Adequate Control Measures: Dengue, Leishmaniasis, and African Trypanosomiasis

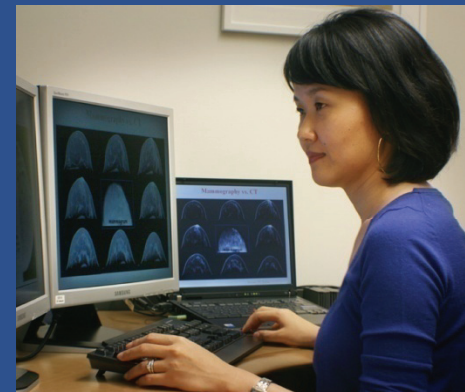
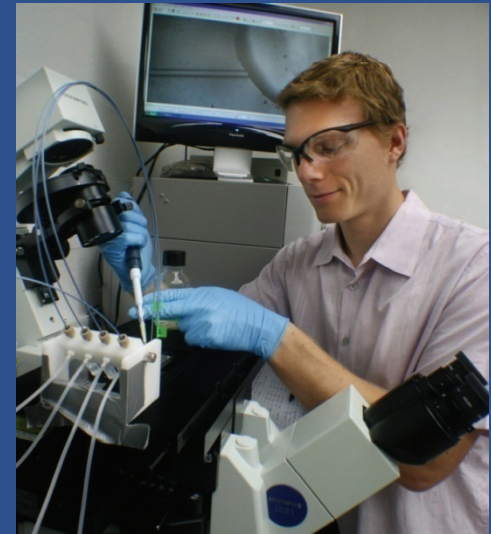


- **Emphasis on global health fits with U.S. emphasis on “soft power”**
- **Scientific advances make an attack on infectious diseases more feasible than ever**
 - RNAi
 - Small molecule screening
 - Genomics
 - Vaccine development
- **Push beyond AIDS, malaria, TB to the long list of neglected tropical diseases (NTDs) that affect hundreds of millions of people worldwide**
- **Include chronic noncommunicable diseases and injuries, now responsible for more than half of the deaths in the developing world**

Opportunity #5: Reinvigorating and empowering the biomedical research community



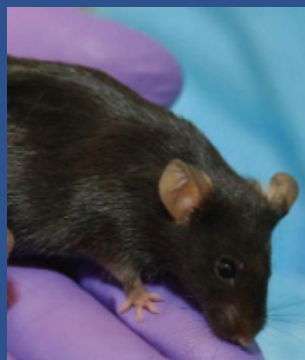
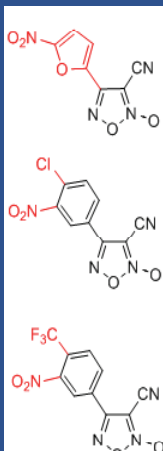
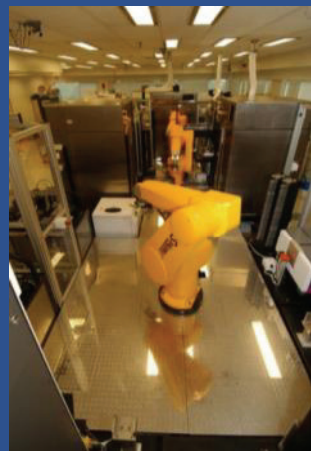
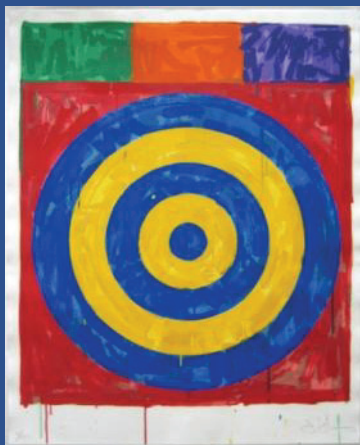
Great projects
outside the box



- **Emphasis on innovation**
 - Pioneer awards
 - New Innovator awards
 - Transformative RO1s
- **Continuing to innovate in peer review**
- **Reinvigorating the Common Fund**
 - Seven new programs in 2010
- **Training and career development programs**
 - Seek to reduce age at first grant award
 - Encouraging underrepresented groups – Pathfinder Award
 - Women in science

Towards a new paradigm in translation ...





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

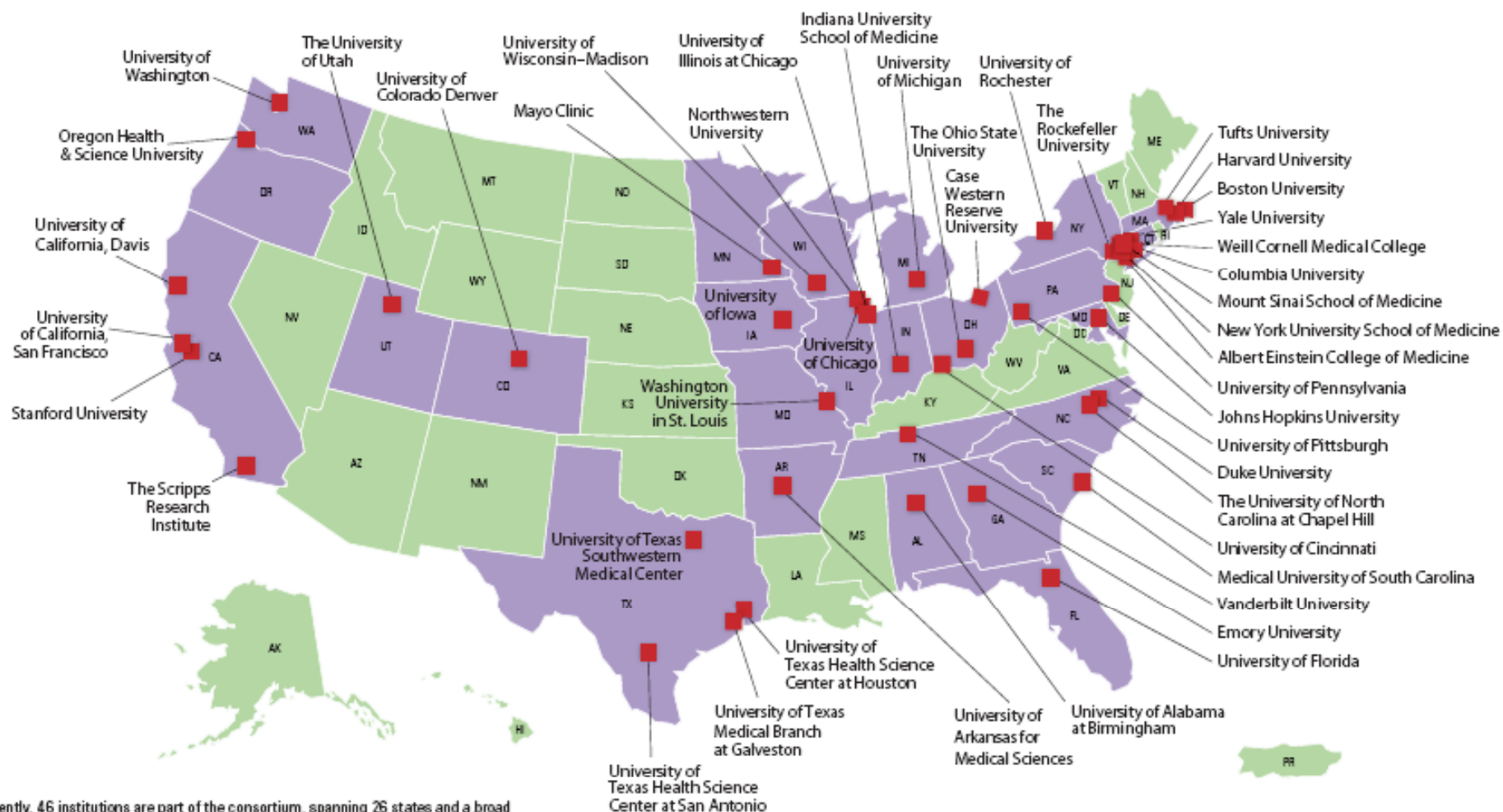
NIH TRND

NIH RAID

Pharma, Biotech, NIH Clinical Center, CTSA's

Clinical and Translational Science Awards

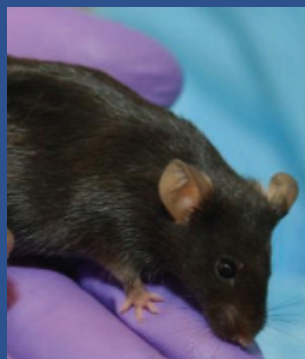
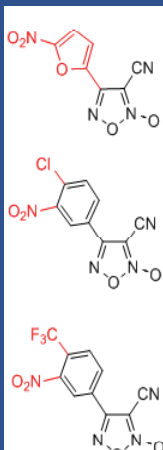
46 CTSA sites in 26 states; 60 sites when fully implemented



Currently, 46 institutions are part of the consortium, spanning 26 states and a broad range of scientific expertise. Consortium members represent each geographic region of the country. When fully implemented, 60 institutions will be linked together to energize the discipline of clinical and translational science.

CTSA Clinical & Translational[®] Science Awards

■ = CTSA institutions (46)
 ■ = CTSA states (26)



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, CTSA

New NIH FDA Partnerships

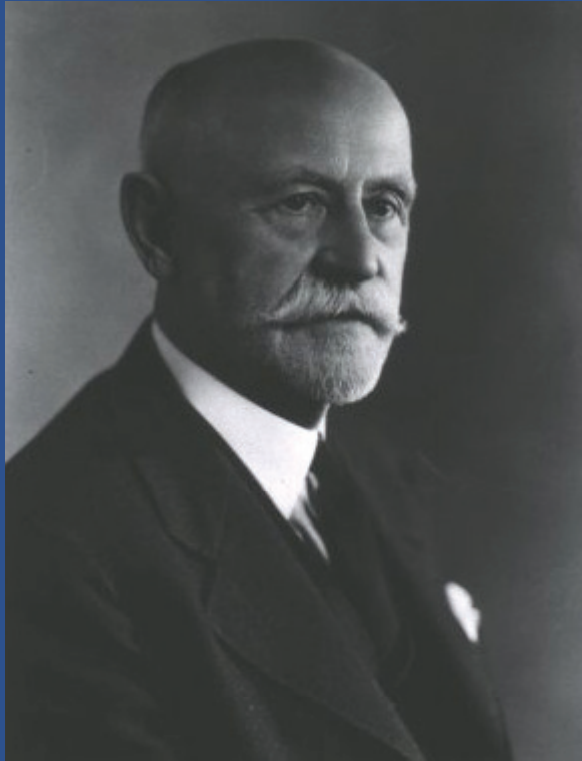
New NIH-FDA Partnership



- 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
- Public Consultation: June 2, 2010



Centenary: 1910 Discovery of Sickle Cell Anemia

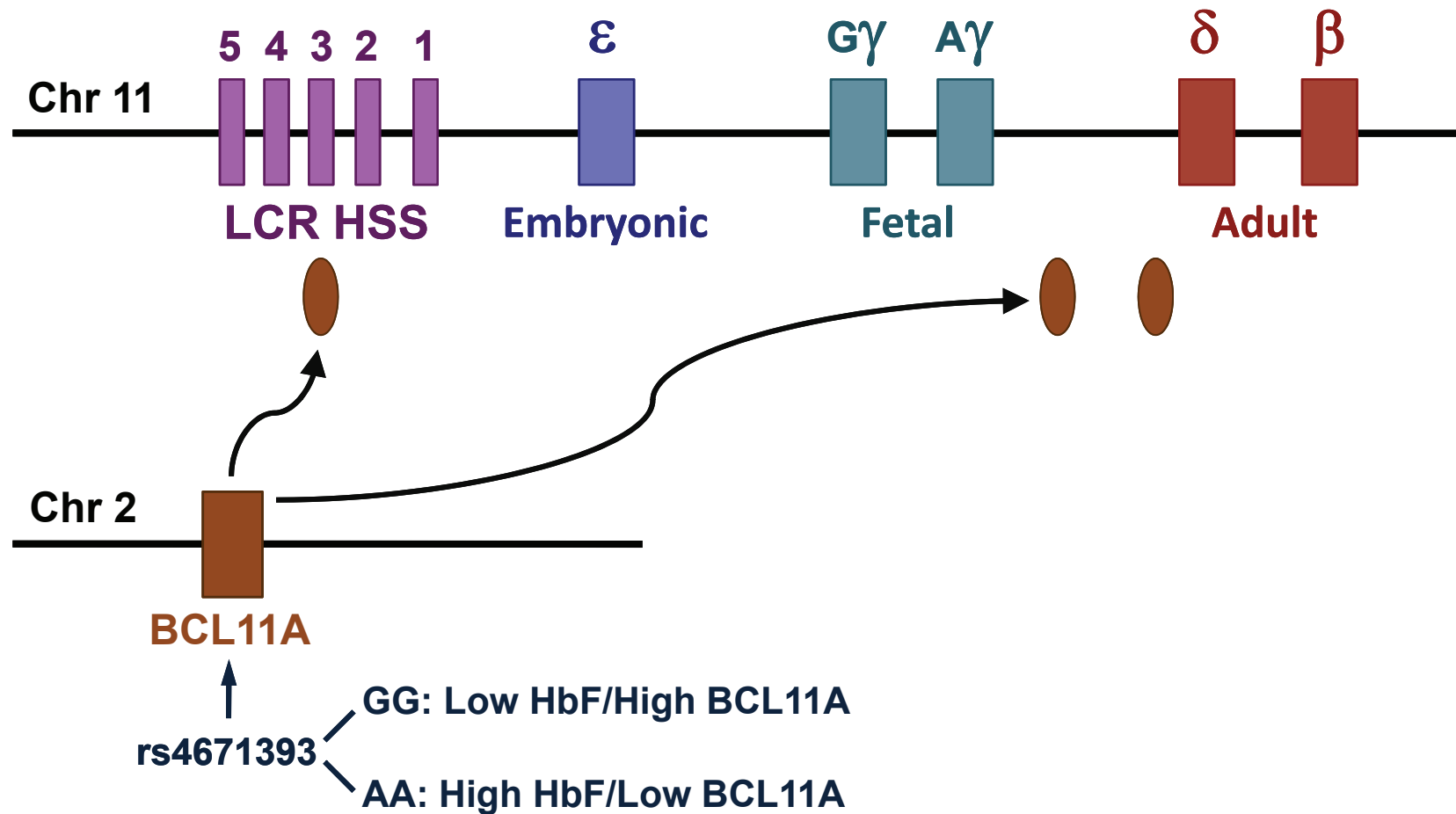


James B. Herrick

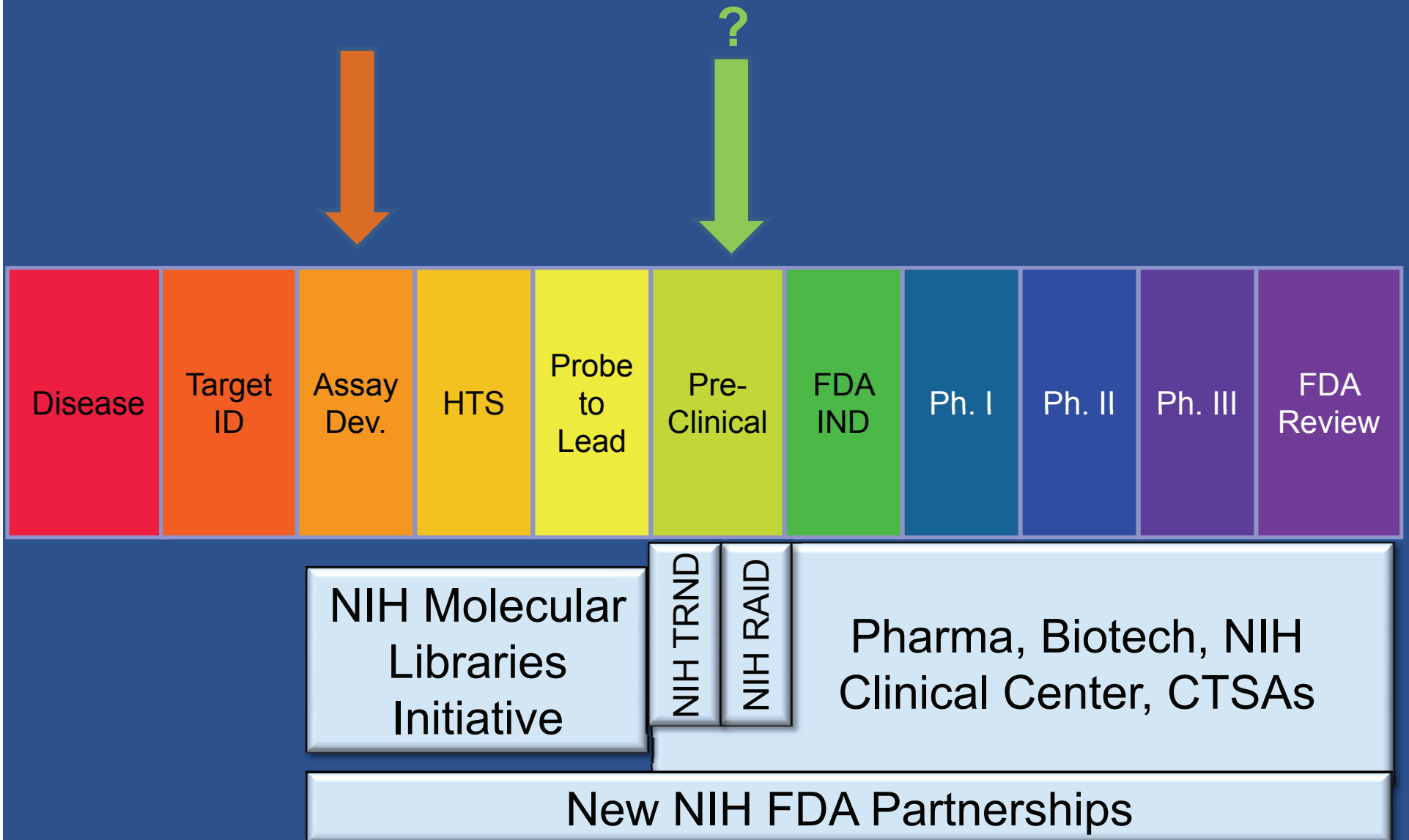
Archives of Internal Medicine (1910) vol. 5
PECULIAR ELONGATED AND SICKLE-SHAPED RED BLOOD
CORPUSCLES IN A CASE OF SEVERE ANEMIA
JAMES B. HERRICK, M.D.



From a Genetic Association to a Genetic Switch: GWAS informs Human Biology and Disease



The New Paradigm for Translation: *Sickle Cell Anemia*



Health Care Reform

An Act

Entitled The Patient Protection and Affordable Care Act.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE; TABLE OF CONTENTS.

(a) **SHORT TITLE.**—This Act may be cited as the “Patient Protection and Affordable Care Act”.

(b) **TABLE OF CONTENTS.**—The table of contents of this Act is as follows:

Sec. 1. Short title; table of contents.

TITLE I—QUALITY, AFFORDABLE HEALTH CARE FOR ALL AMERICANS

Subtitle A—Immediate Improvements in Health Care Coverage for All Americans

Sec. 1001. Amendments to the Public Health Service Act.

“PART A—INDIVIDUAL AND GROUP MARKET REFORMS

“SUBPART II—IMPROVING COVERAGE

“Sec. 2711. No lifetime or annual limits.

“Sec. 2712. Prohibition on rescissions.

“Sec. 2713. Coverage of preventive health services.

“Sec. 2714. Extension of dependent coverage.

“Sec. 2715. Development and utilization of uniform explanation of coverage documents and standardized definitions.

“Sec. 2716. Prohibition of discrimination based on salary.

“Sec. 2717. Ensuring the quality of care.

“Sec. 2718. Bringing down the cost of health care coverage.

“Sec. 2719. Appeals process.

Sec. 1002. Health insurance consumer information.

Sec. 1003. Ensuring that consumers get value for their dollars.

Sec. 1004. Effective dates.

Subtitle E—Immediate Actions to Preserve and Expand Coverage

Sec. 1101. Immediate access to insurance for uninsured individuals with a pre-existing condition.

Sec. 1102. Reinsurance for early retirees.

Sec. 1103. Immediate information that allows consumers to identify affordable coverage options.

Sec. 1104. Administrative simplification.

Sec. 1105. Effective date.

Subtitle C—Quality Health Insurance Coverage for All Americans

PART I—HEALTH INSURANCE MARKET REFORMS

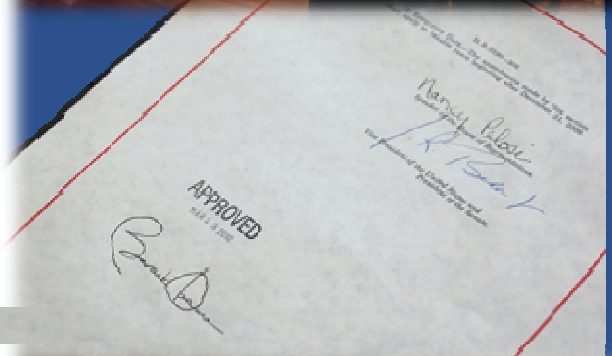
Sec. 1201. Amendment to the Public Health Service Act.

“SUBPART I—GENERAL REFORM

“Sec. 2704. Prohibition of preexisting condition exclusions or other discrimination based on health status.

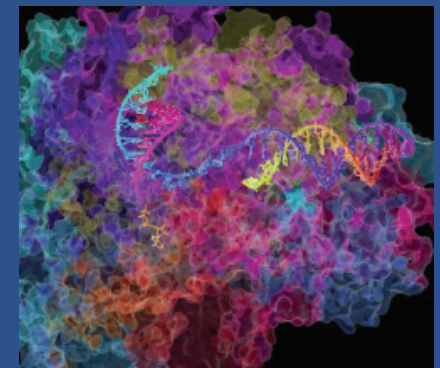
“Sec. 2701. Fair health insurance premiums.

“Sec. 2702. Guaranteed availability of coverage.



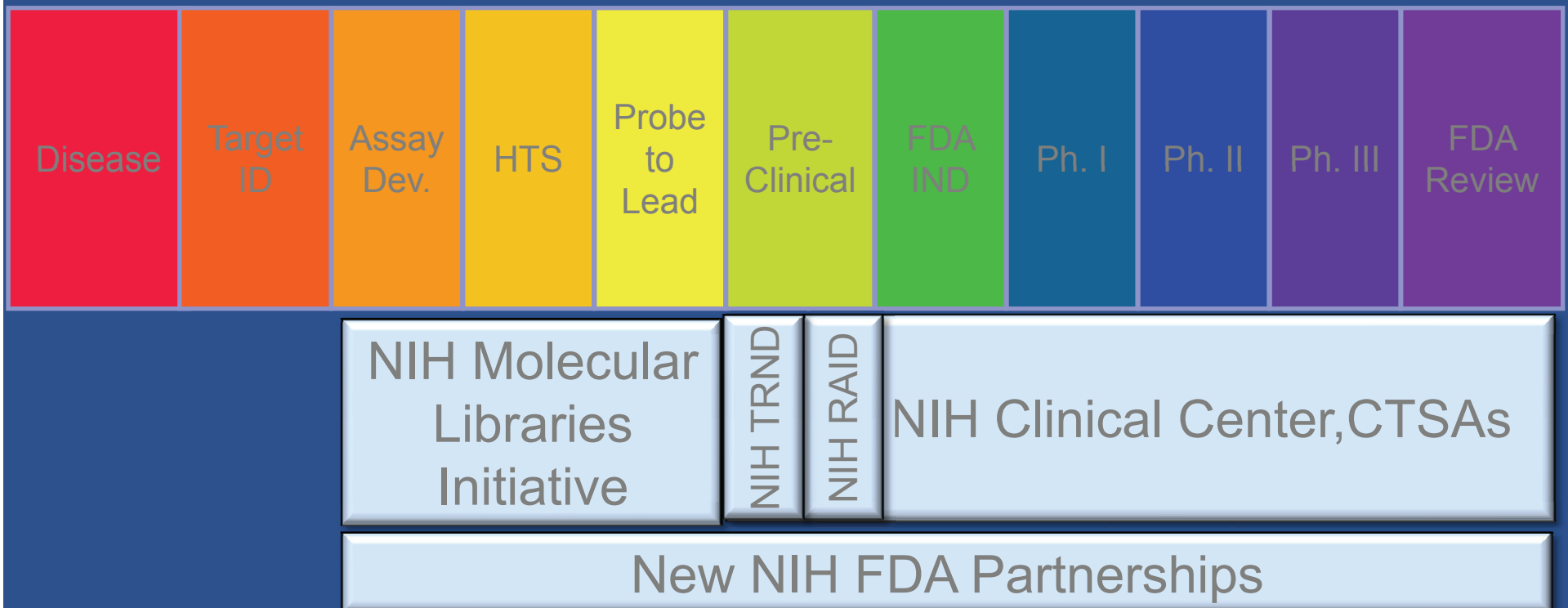
A Bold New Feature of the Health Care Bill: The Cures Acceleration Network (CAN)

- Goal: dramatically advance development of new treatments and cures for debilitating and life-threatening diseases by reducing barriers between laboratory discoveries and clinical trials
- Authorized budget for FY 2010: \$500M
- 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



CAN provides a framework for integration *And for process engineering*

Cures Acceleration Network



Making the Case for Research

- Educate others about the importance of biomedical research
- Inspire passion for science in next generation
- Contact NIH with suggestions, comments
- *Continue to encourage innovation*

NIH-LISTENS@nih.gov



Curiosity Creates Cures
The Value and Impact of Basic Research



Bring Exciting Hands-On Learning to Your Area

It's more than just a day. We're building a community.



TEACHERS

CREATE PROJECT REQUESTS TO FIND RESOURCES. SIGN UP NOW.



SCIENTISTS & TECHIES

WE'LL MATCH YOU TO PROJECTS NEEDING YOUR HELP. SIGN UP NOW.



VOLUNTEERS

NOT A SCIENTIST OR TECHIE BUT STILL WANT TO HELP? SIGN UP NOW.



ORGANIZATIONS

PROVIDE THE OPPORTUNITY FOR YOUR ORGANIZATION TO JOIN OUR ONLINE COMMUNITY. SIGN UP NOW.



Career Speaker Series

South Gate, CA



Lab Improvement


Tempe, AZ



View a map of all projects

What is National Lab Day?

What can I do?

Follow us on  Twitter

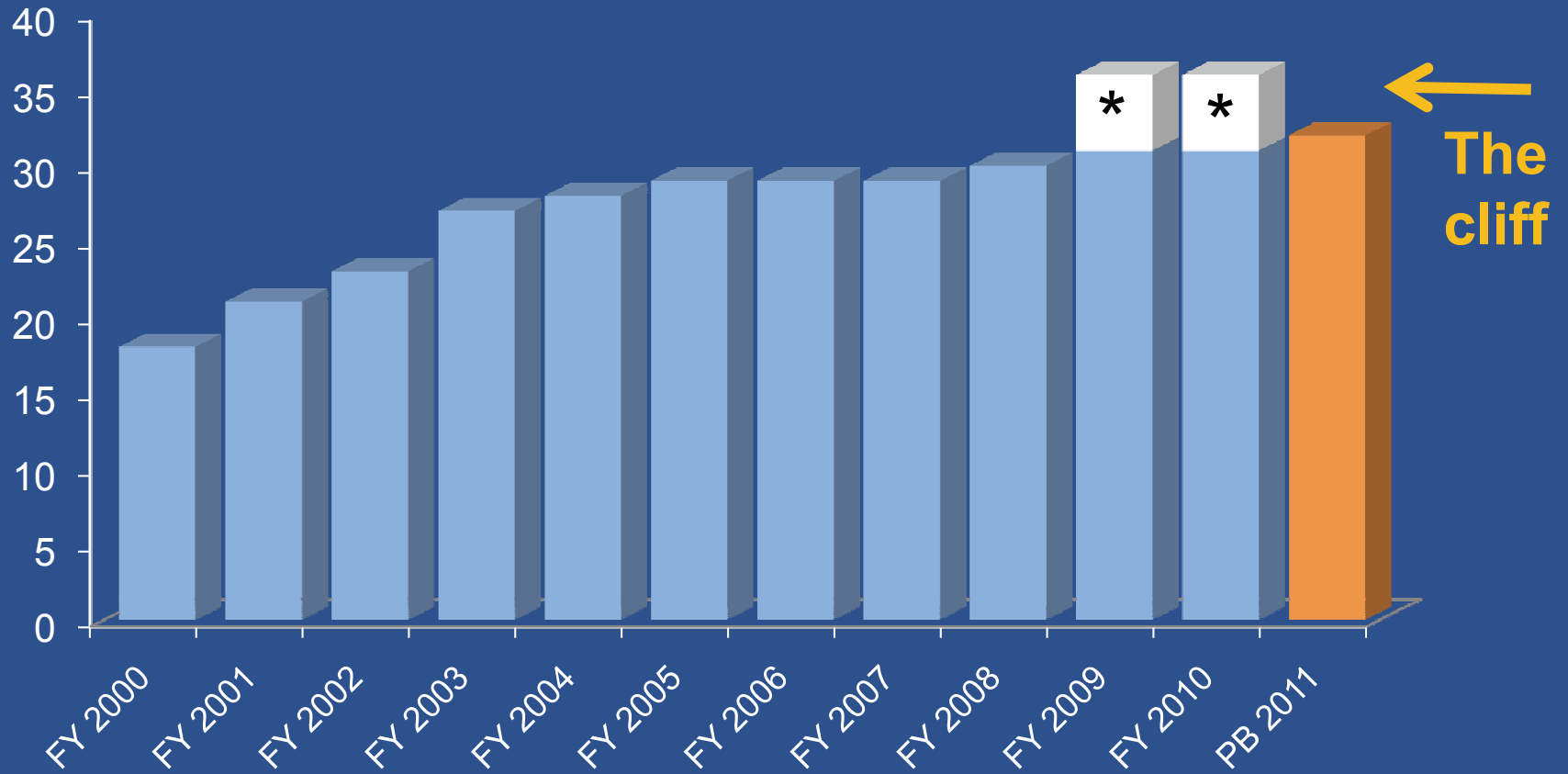
Sponsors



"Lifting American students from the middle to the top of the pack in STEM achievement over the next decade will not be attained by government alone" ~ President Obama

NIH FY 2011 President's Budget Request \$32.2 Billion

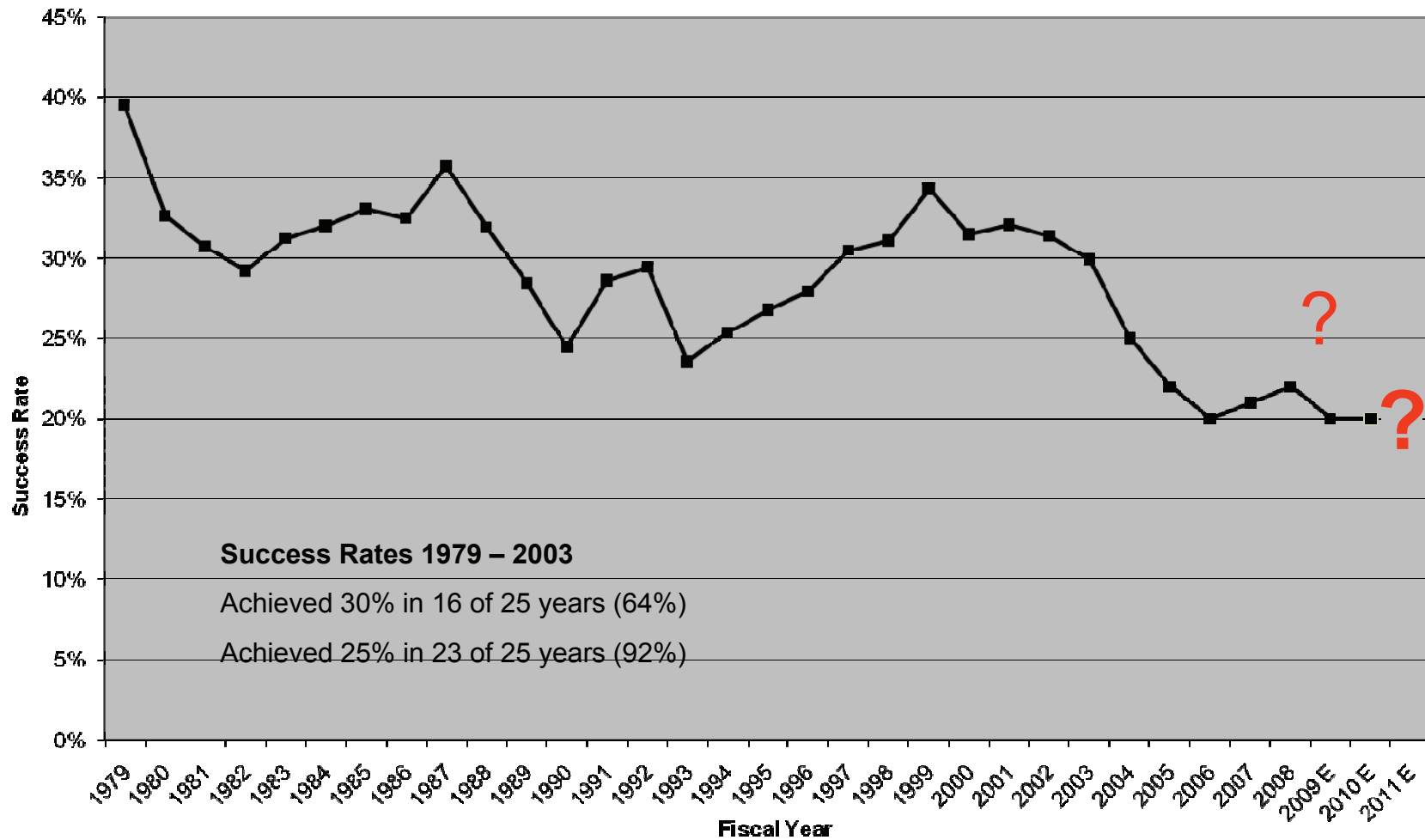
Increase of \$1B or 3.2% over FY 2010 non-ARRA dollars



*ARRA Funding: \$5B for FY 2009, \$5B for FY 2010

Annual levels represent total budget authority, including special type 1 diabetes appropriation

NIH Success Rates (1979-2010)



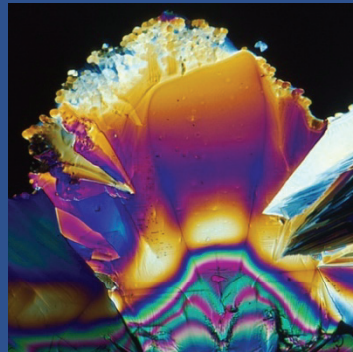
One Voice ...





To wrest from nature the secrets which have perplexed philosophers in all ages, to track to their sources the causes of disease, to correlate the vast stores of knowledge, that they may be quickly available for the prevention and cure of disease — these are our ambitions.

Sir William Osler



NIH

*Turning discovery
into health*

