

A Conversation with NCI Acting Director, Dr. Douglas R. Lowy



NATIONAL CANCER INSTITUTE
Office of Advocacy Relations

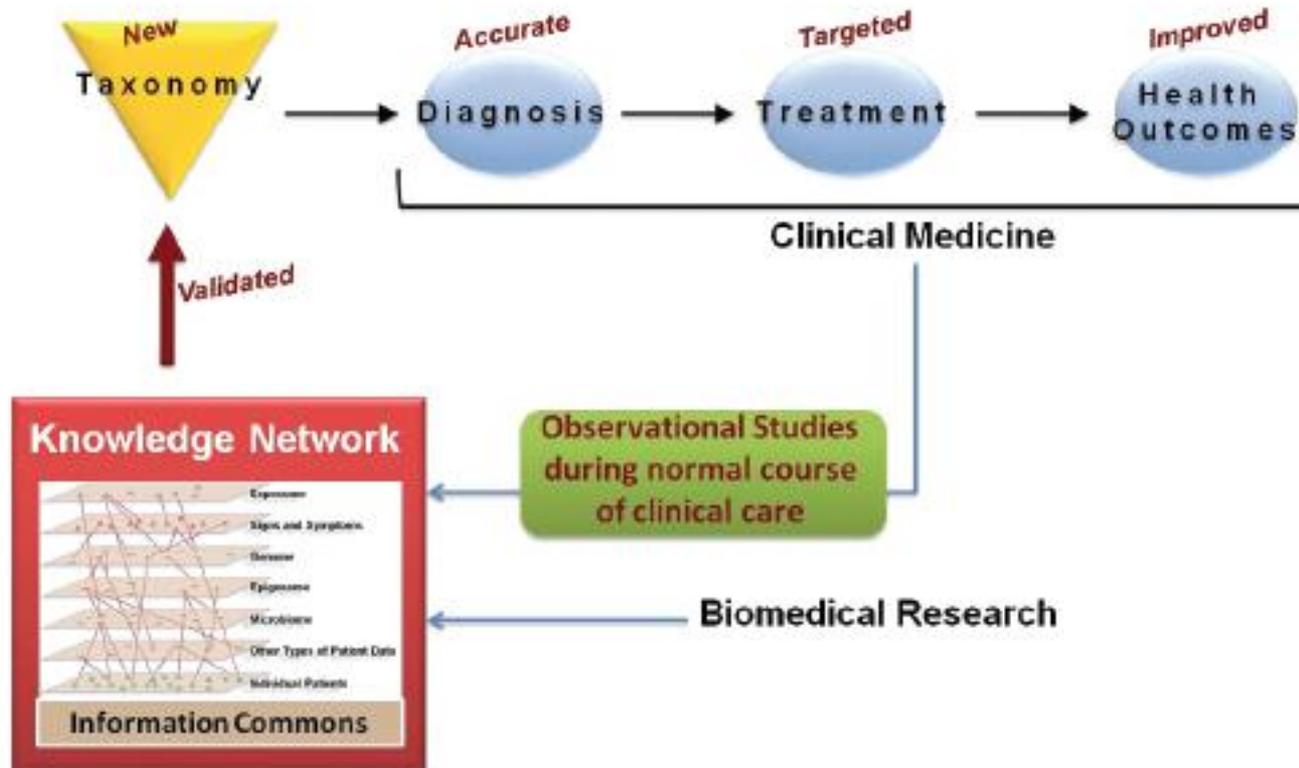
Webinar
Monday, June 1, 2015
1:00 PM EDT

What is Precision Medicine?

Toward Precision Medicine

Building a Knowledge Network for Biomedical Research
and a New Taxonomy of Disease

NATIONAL RESEARCH COUNCIL
OF THE NATIONAL ACADEMIES



“Precision (personalized) medicine refers to the tailoring of medical treatment to the individual characteristics of each patient... Preventive or therapeutic interventions can then be concentrated on those who will benefit, sparing expense and side effects for those who will not.”

Toward Precision Medicine, 2011

This definition inadvertently appears to exclude population-wide public health recommendations, even those based on a mechanistic understanding of disease.

A Modified Definition of Precision Medicine

Interventions to prevent, diagnose, or treat a disease (e.g., cancer), based on a molecular and/or mechanistic understanding of the causes, pathogenesis, and/or pathology of the disease.

Where the individual characteristics of the patient are sufficiently distinct, interventions can be concentrated on those who will benefit, sparing expense and side effects for those who will not.



Precision Medicine
trials to improve
patient outcomes: NCI
initiatives begun since
2014

Precision Medicine Studies Opened in the Past Year and Accruing Nationally in NCTN/NCORP*

- Lung-MAP (Lung squamous cell Cancer Master Protocol)
- ALCHEMIST (Adjuvant Lung Adenocarcinoma Cancer Enrichment and Sequencing Trials)
- MPACT (Molecular Profile based Assignment of Cancer Therapeutics)
- Exceptional Responders: excellent intake of patients from entire cancer community--sequencing efforts underway

**NCTN – National Clinical Trial Network*

**NCORP – National Clinical Oncology Research Program*

NCI-MATCH

Molecular Analysis for Therapy Choice

- Largest, most rigorous precision oncology trial
- Foundational discovery treatment trial, focuses on molecular abnormalities, not on site of tumor origin
- Validated and standardized gene sequencing and selection of targeted therapy (MATCHBOX)
- Trial co-developed by NCI and ECOG-ACRIN, part of NCTN
- Available with NCORP to 2,400 sites nationwide

Adult MATCH trial

- Current clinical trial paradigm: evaluate drugs in a particular cancer type at a particular organ site
- Adult MATCH: Follows a different paradigm: based on targeted treatment of specific “actionable” molecular abnormalities in cancers independent of the organ site
 - A public-private partnership with ~20 pharmaceutical companies; testing a range of targeted treatments in a single trial (off-label use of FDA-approved drugs or investigational drugs)

Pediatric MATCH

- For children with advanced cancers that have progressed on standard therapy
- Modeled after Adult MATCH, DNA sequencing will be used to identify children whose tumors have a genetic abnormality for which either an approved or investigational targeted therapy exists
- A partnership with the NCI-funded Children's Oncology Group, Pediatric MATCH is still under development but a timetable for launch or patient enrollment will be available soon

The Precision Medicine Initiative (PMI)

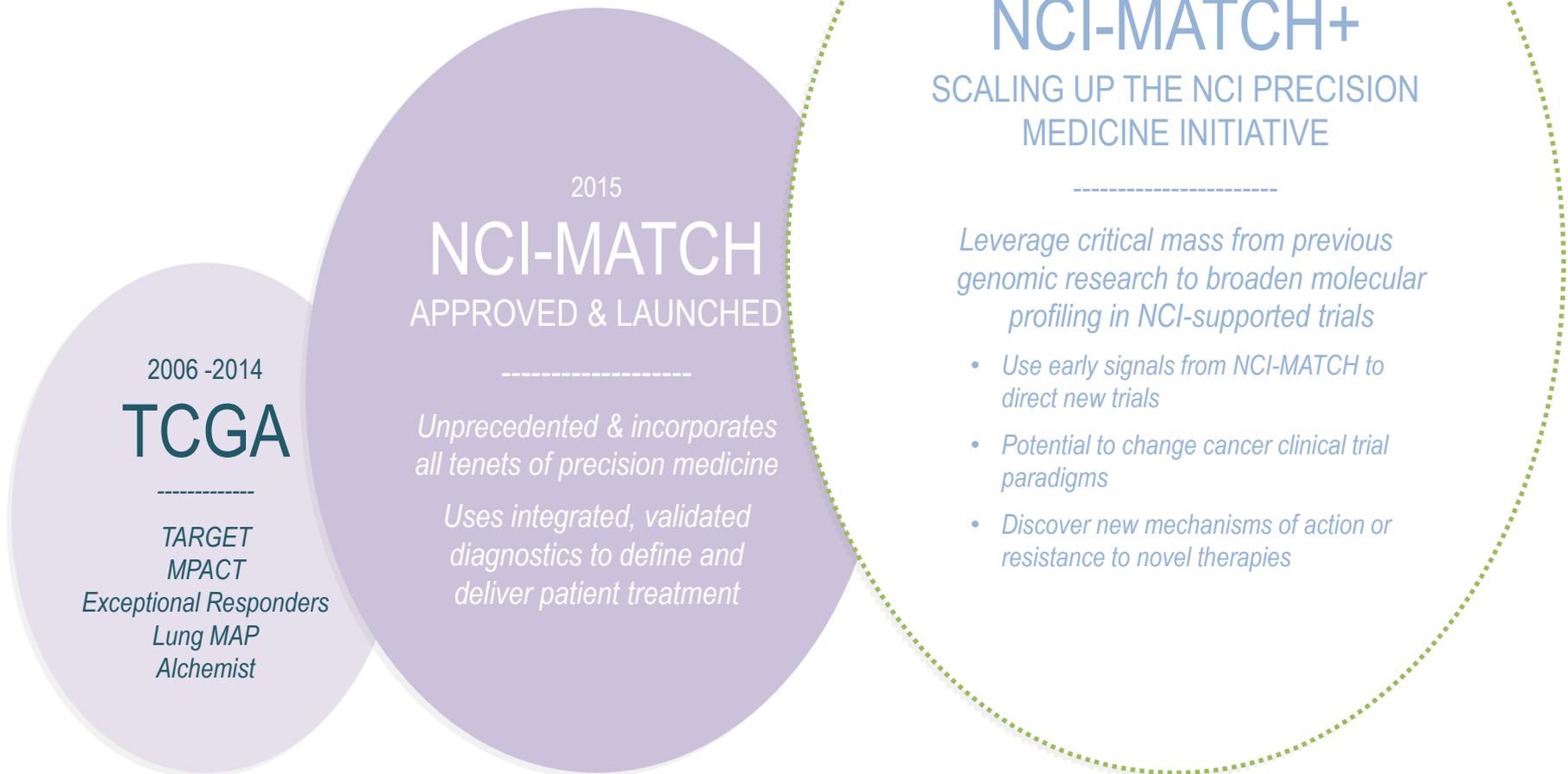
The Precision Medicine Initiative: Part of the President's FY16 Budget Proposal

- The president's PMI proposal has 4 parts:
 - ***Expand the application of precision medicine in cancer treatment (NCI, \$70 million)***
 - Establish a cohort of 1 million or more volunteers to help bring precision medicine to a wide range of diseases (NIH, \$130 million)
 - Increase FDA's capacity to support the advance of precision medicine in regulatory issues (FDA, \$10 million)
 - Develop interoperability standards for data exchange across systems while protecting privacy (ONC, \$5 million)

ONC: Office of the National Coordinator for Health Information Technology

Funding of PMI subject to Congressional appropriations

The Promise of Precision Oncology



The NCI Precision Medicine Initiative: MATCH+

- \$70 million requested by President Obama to expand genomics-based clinical and pre-clinical studies:
 - Genomic master protocol (MATCH) trials of adult and pediatric cancers
 - Repository of patient-derived preclinical models for evaluating targeted therapeutics
 - Drug resistance: understand & overcome with combination therapy
 - National cancer database to integrate genomic information with clinical response and outcome

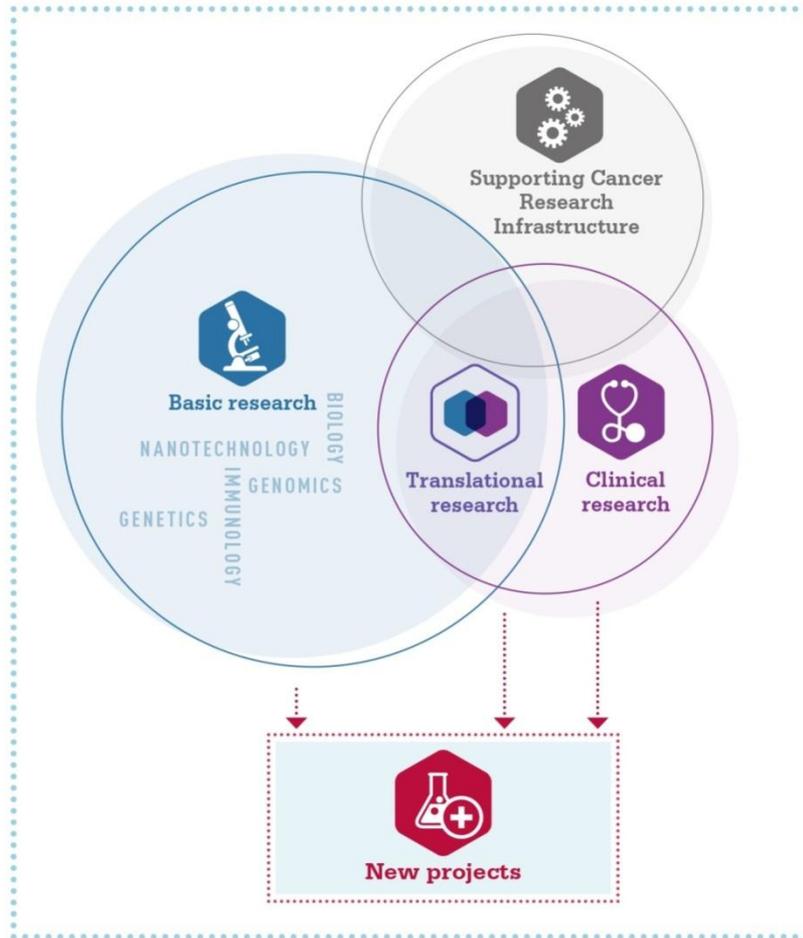


NCI Budget FY2015 and FY2016: How it Works

The Federal Budget Cycle

- President Issues Budget Request to Congress (February)
- Congressional Action
 - Budget Committees produce annual budget resolutions to set overall funding levels
 - House and Senate Appropriations Committees develop allocations for each of the 12 subcommittees
 - Subcommittees determine agency appropriations (NIH is funded via the Labor-HHS-Education subcommittee)
- *NIH funding subject to Congressional appropriations process – levels may be higher or lower than PB request*

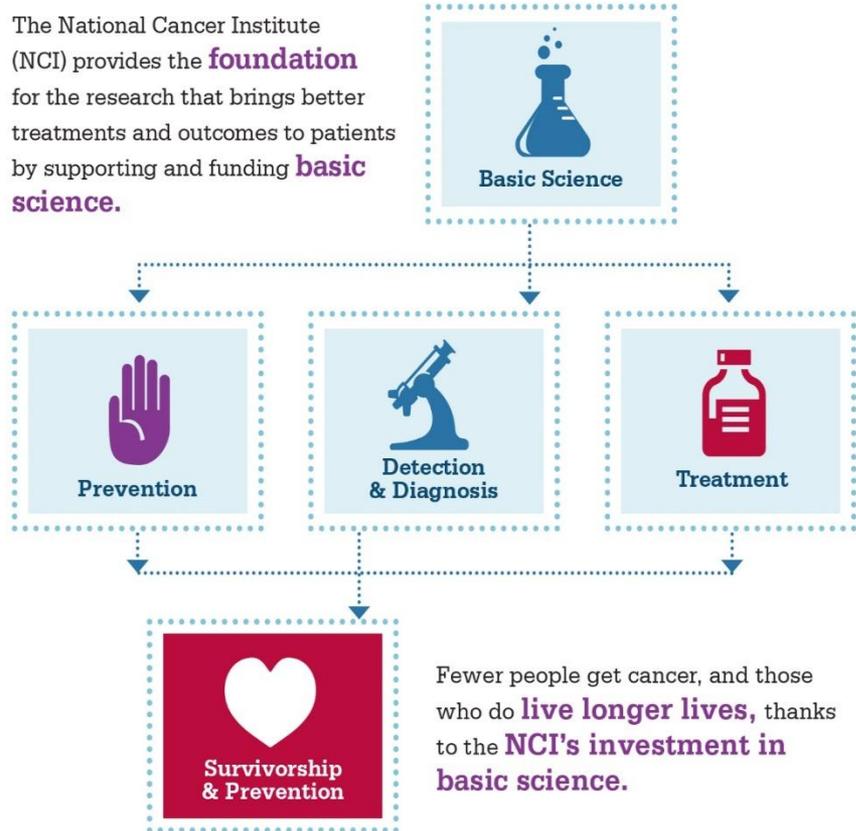
Ongoing and long-term projects, infrastructure, and training programs make up more than 80% of the annual budget.



Each year only a small portion of the budget is available for new projects.

Funding basic science leads to discoveries that are needed to advance cancer research.

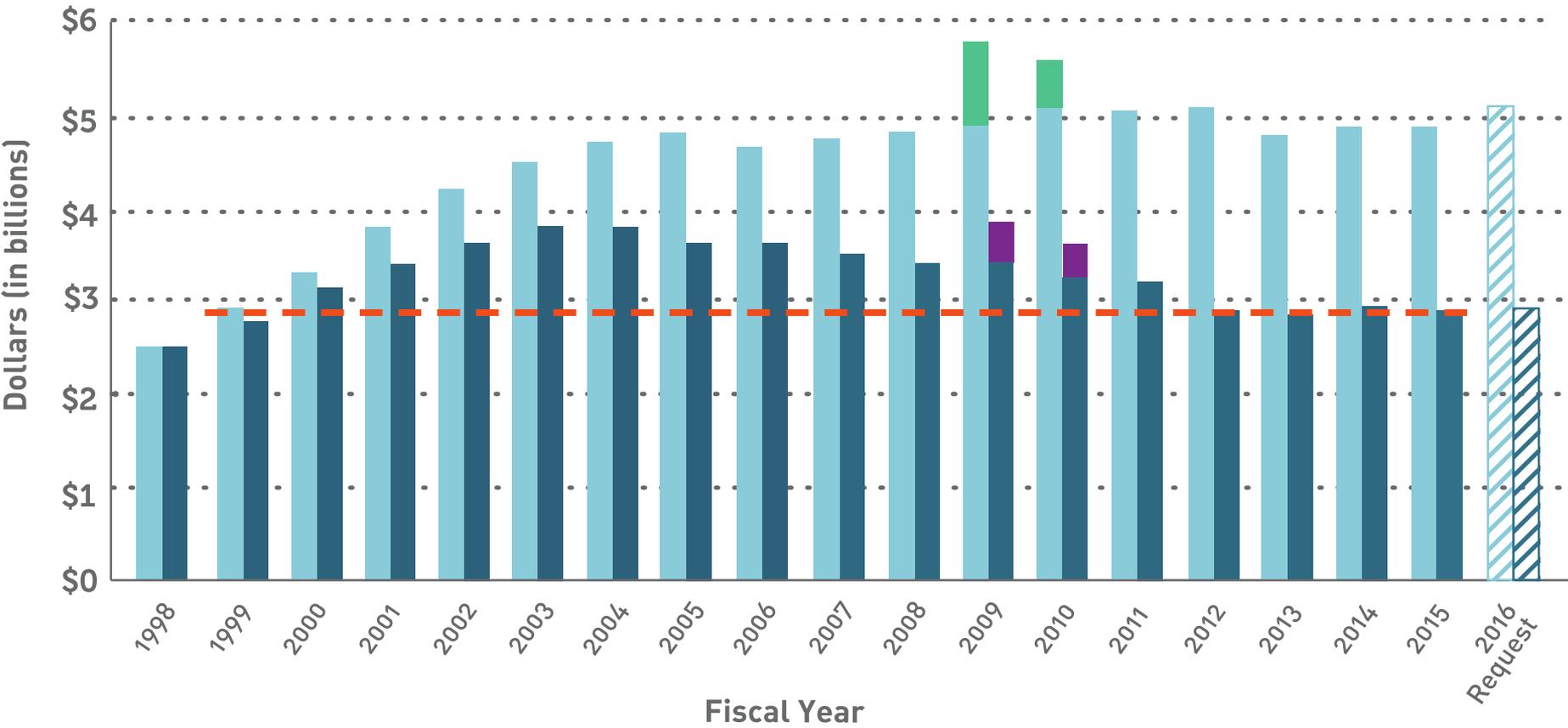
The National Cancer Institute (NCI) provides the **foundation** for the research that brings better treatments and outcomes to patients by supporting and funding **basic science**.



Fewer people get cancer, and those who do **live longer lives**, thanks to the **NCI's investment in basic science**.

About 48 percent of NCI's budget supports basic research

NCI BUDGET 2005-2015: A DECADE OF LEVEL BUDGETS & PROGRESSIVELY DECREASING PURCHASING POWER

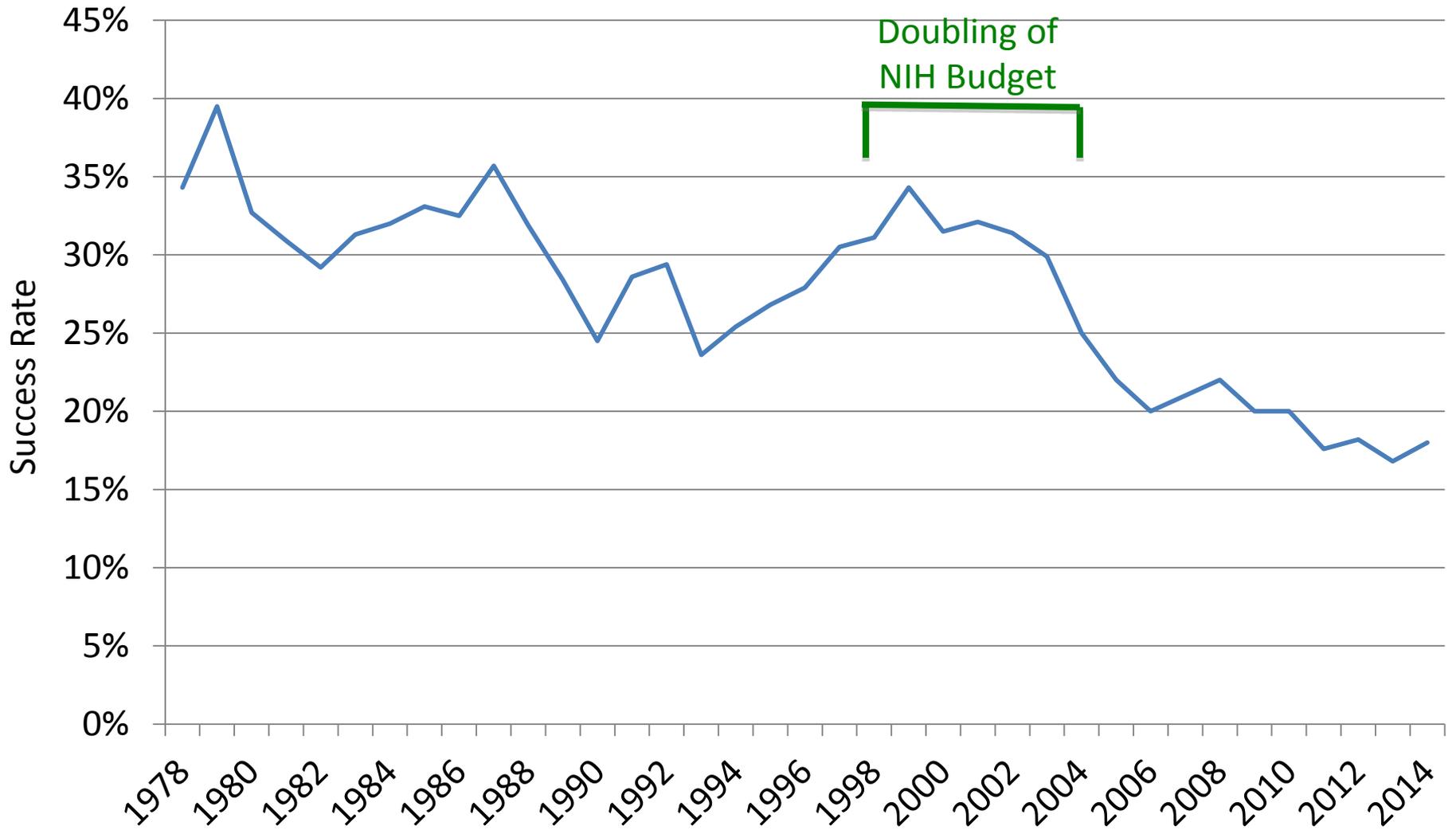


■ NCI Budget
 ■ NCI Budget Adjusted for Inflation (FY 1998 dollars)
 ■ ARRA Funding (Public Law 111-5)
 ■ ARRA in 1998 Dollars

- - - - - The dashed line at \$2.9 billion indicates that the inflation-adjusted 2015 budget was similar to the 1999 budget, the first year of the “NIH doubling”

Grant Success Rates

FY 1978 – 2014



The President's FY16 Budget Proposal for NIH and NCI

- A \$1 billion increase for NIH (from \$30.084 billion to \$31.084 billion)
- A \$145 million increase for NCI (from \$4.953 billion to \$5.098 billion)

**subject to Congressional appropriations – levels may be higher or lower than PB request*

Summary

- Precision medicine represents a rational approach to cancer prevention, screening, and treatment
- The Precision Medicine Initiative will enable the field of predictive oncology, whose goal is to deliver the right drug(s) to the right patient