Funding Large Research Studies

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Recommendation 4.3

The Committee recommends that the Secretary of HHS, working with the Secretaries of Veterans Affairs and Defense, develop a public-private partnership with drug sponsors, public and private insures, for profit and not for profit health care provider organizations, consumer groups, and large pharmaceutical companies to prioritize, plan and organize funding for confirmatory drug safety and efficacy studies of public health importance. Congress should capitalize the public share of this partnership.

Funding Large Studies in the Public Interest : 2 Things Need to Happen

- **Those with a stake in the game need to "belly up to the bar"**
  - Industry—participate (your costs will be reduced)
  - HHS 1– quit passing the buck and come up with a plan
    - NIH-not into "coke vs pepsi"; AHRQ—broke; CMS—not its mission; VA-focused on one population
  - Health plans– talk to action ratio is infinity for 30 years
  - AMC's—reward those who do it
  - Consumer groups—keep pushing
- **Using NIH money as a "chemoattractant" will yield great benefits in workforce, standardization and interoperability**
- **We need to modernize the horribly inefficient clinical research system to rid it of wasteful spending**

Key Quotes

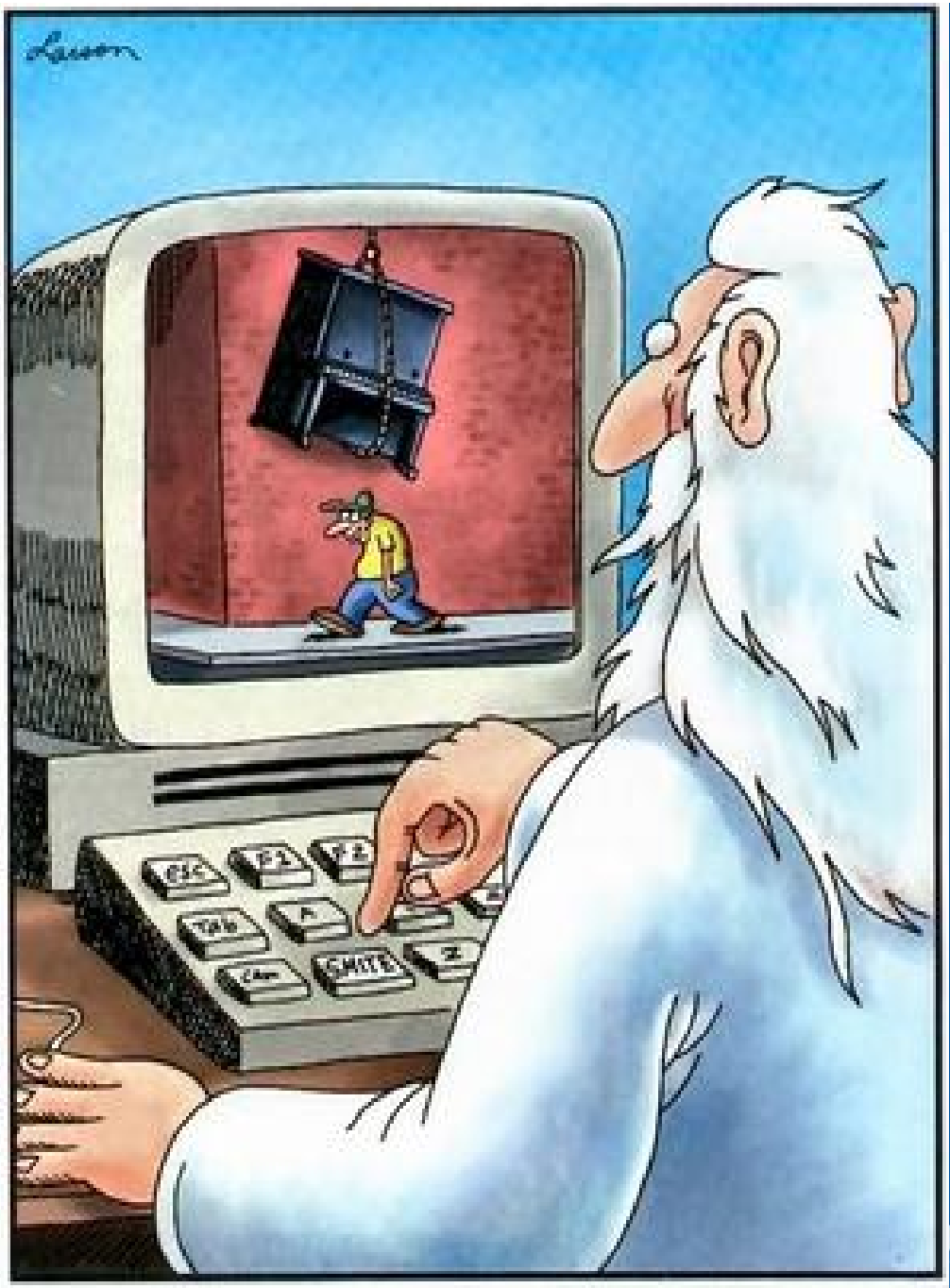
- **Ron Krall**

  If we don't develop a public private partnership, every government agency, health system and company will develop its own system

- **Mark McClellan**

  The total cost of a public private partnership will be less than the total cost of each entity developing its own system

- **My takeaway: A federated public private partnership model with a common informatics (data standards and nomenclature) model is the only way to go!**



God at His computer

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Inescapable Facts

- **Premarketing studies cannot assure a positive balance of safety and effectiveness for chronic treatments**
- **Electronic health records will amplify the signals of risk without necessarily clarifying the benefits**
- **There is no single "cookie-cutter" template for doing clinical research**
- **Large numbers of heterogeneous subjects/patients must be evaluated for long periods of time**

  Even with pharmacogenomics, many more subpopulations will be identified leading to the need to study more people

  20 small studies of different types of diabetes will take more pts than a single large trial, especially when multiple testing is considered

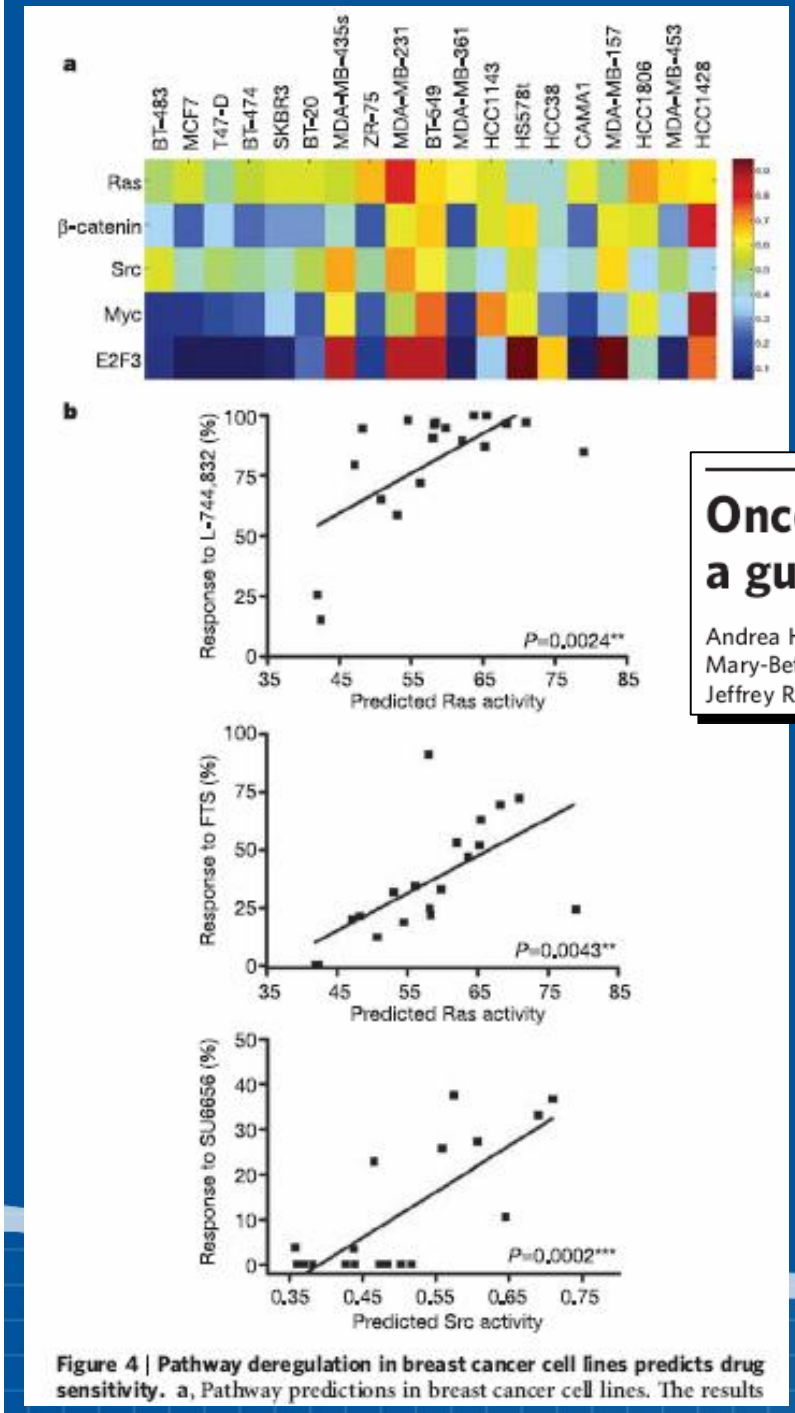


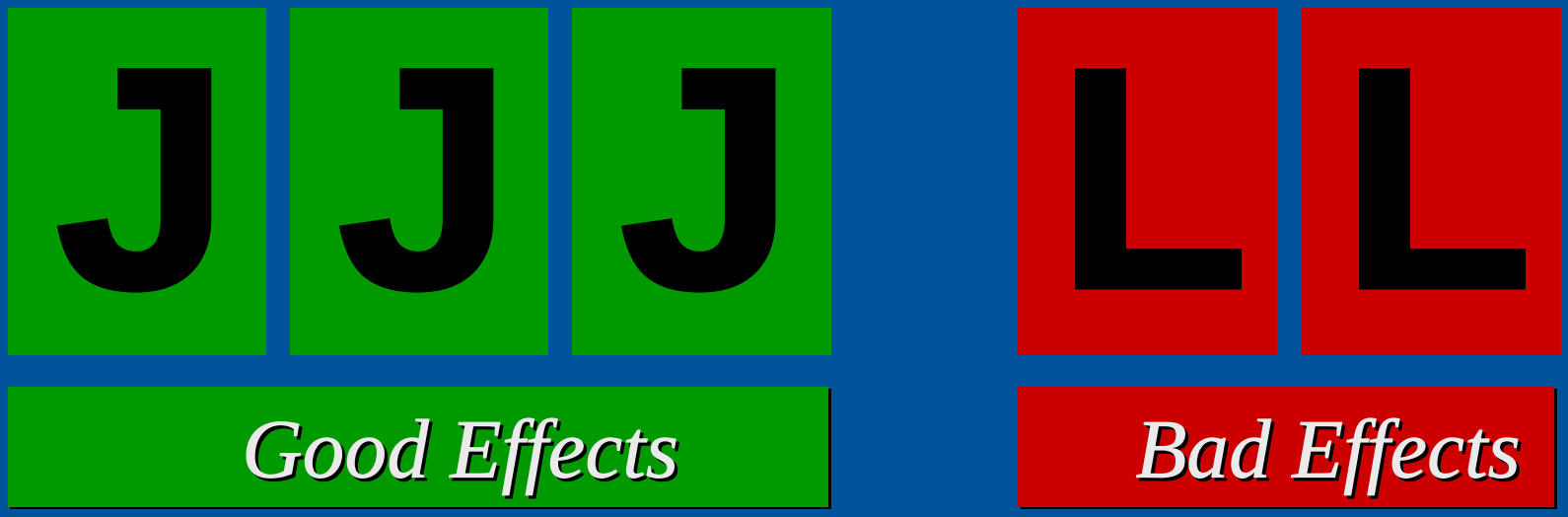
Figure 4 | Pathway deregulation in breast cancer cell lines predicts drug sensitivity. a, Pathway predictions in breast cancer cell lines. The results

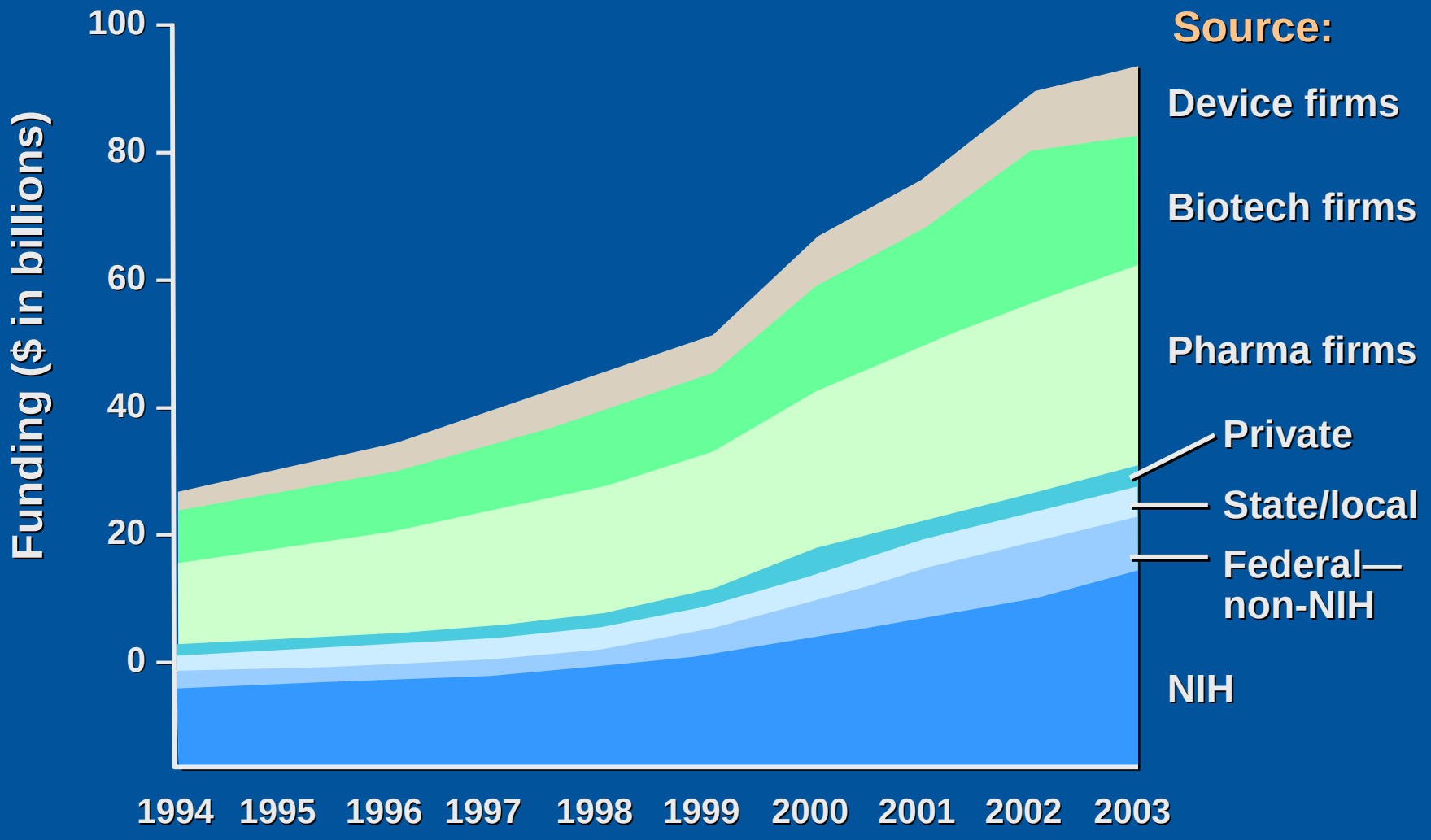
Oncogenic pathway signatures in human cancers as a guide to targeted therapies

Andrea H. Bild[1,2], Guang Yao[1,2], Jeffrey T. Chang[1,2], Quanli Wang[1], Anil Potti[1,4], Dawn Chasse[1,2], Mary-Beth Joshi[3], David Harpole[3], Johnathan M. Lancaster[7], Andrew Berchuck[5], John A. Olson Jr[1,3], Jeffrey R. Marks[3], Holly K. Dressman[1,2], Mike West[6] & Joseph R. Nevins[1,2]

Bild et al. Nature. 2006 Jan 19;439 (7074):353-7.

Therapies Always Cause a Combination of:





Reproduced from Moses et al., JAMA 2005;294:1333-42

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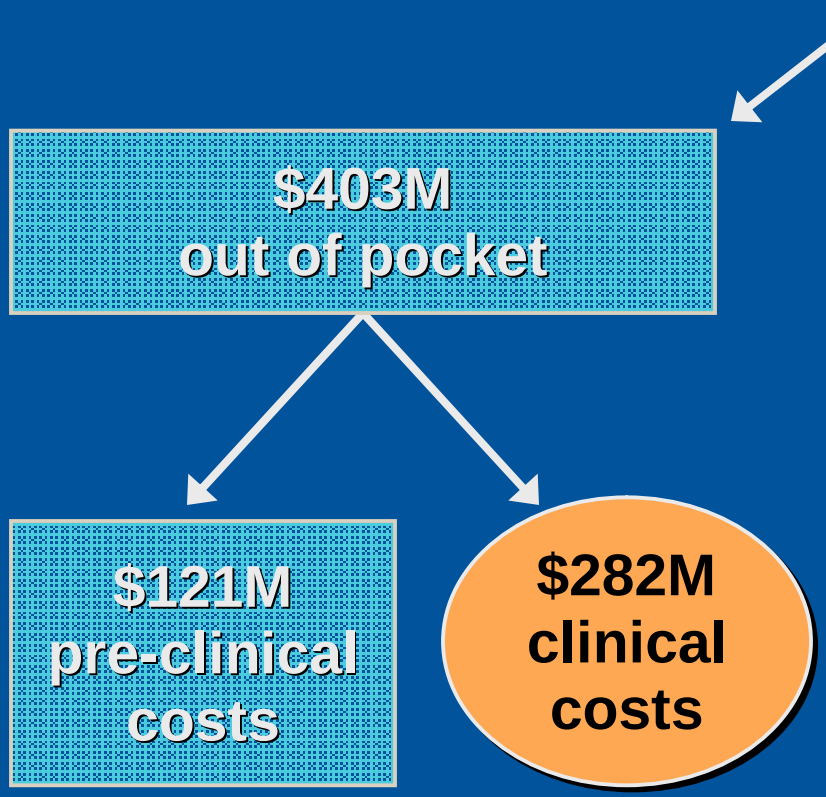
Transforming Medicine

Cost of Drug Development: $802 million

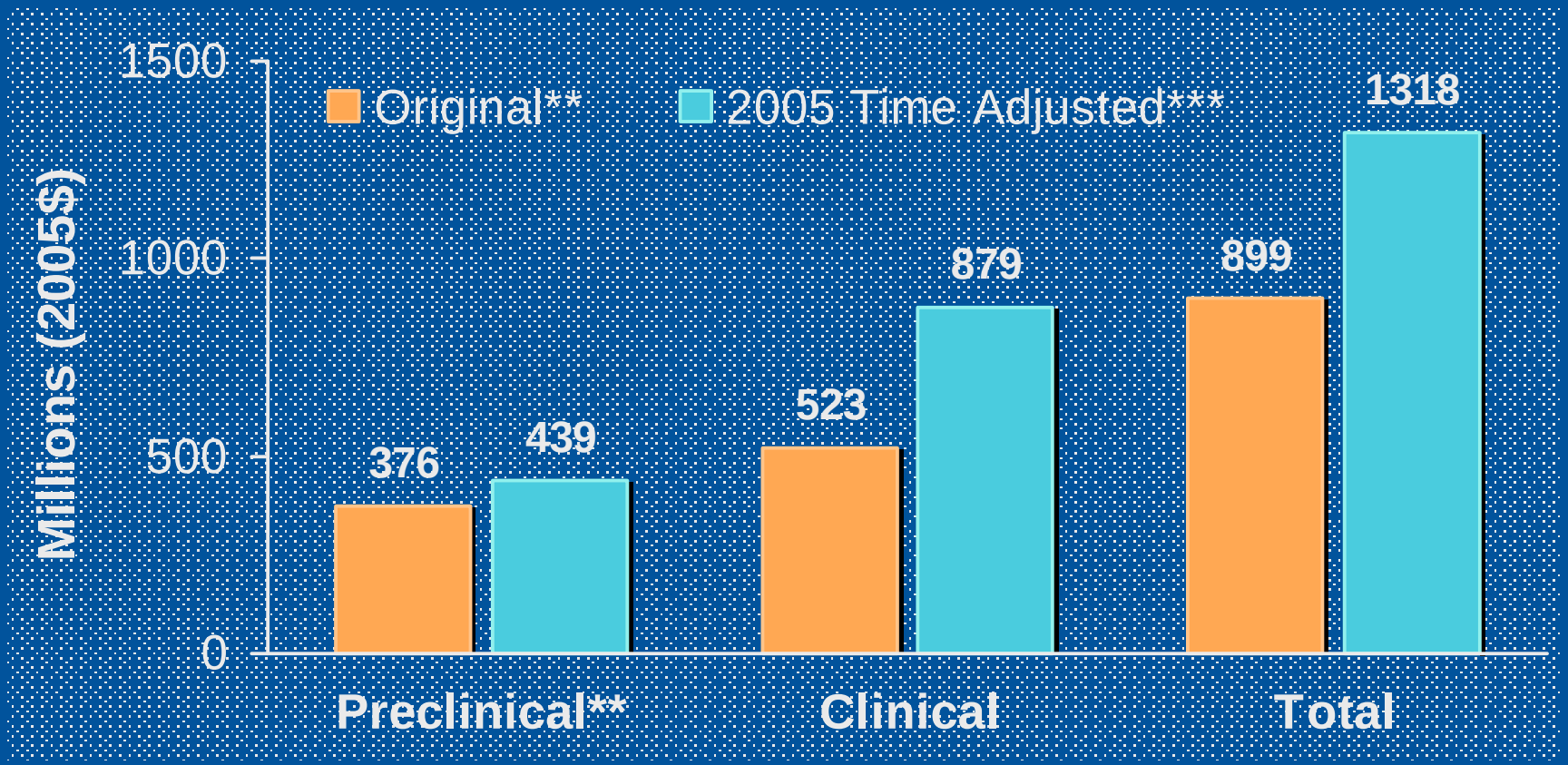


**Assumes discount rate of 11% to time of market approval.*

…and increasing

DeMasi, Hansen and Grabowski, Jl of Health Economics 2003

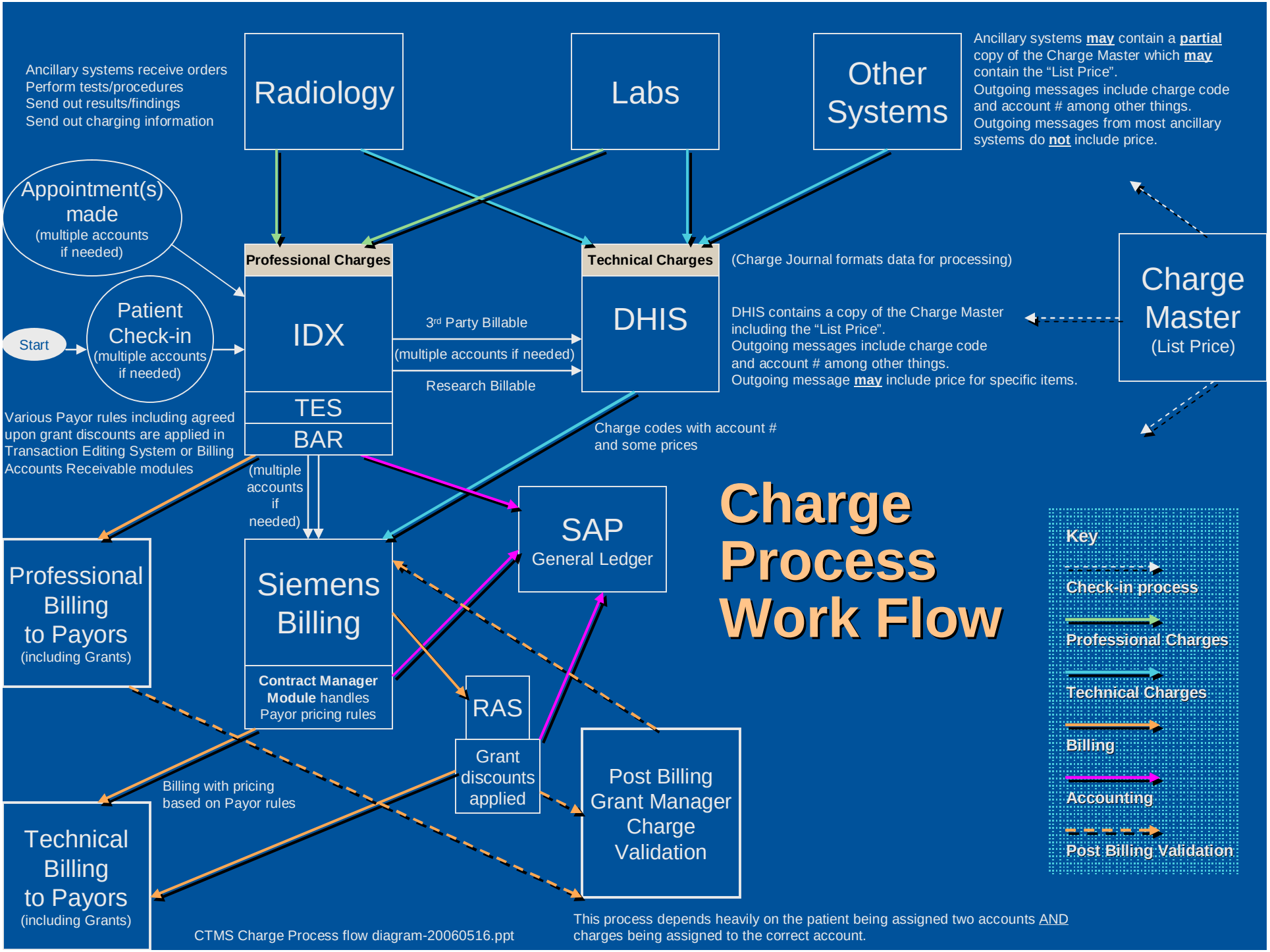
Comparative Pre-Approval Capitalized Costs per Approved New Molecule



*** All R&D costs (basic research and preclinical development) prior to initiation of clinical testing*

**** Based on a 5-year shift and prior growth rates for the preclinical and clinical periods*

DiMasi et al. 2007


Ancillary systems receive orders
Perform tests/procedures
Send out results/findings
Send out charging information
Radiology
Labs
Other Systems
Ancillary systems may contain a partial copy of the Charge Master which may contain the "List Price".
Outgoing messages include charge code and account # among other things.
Outgoing messages from most ancillary systems do not include price.
Appointment(s) made
(multiple accounts if needed)
Patient Check-in
(multiple accounts if needed)
Start
Professional Charges
IDX
TES
BAR
Technical Charges
DHIS
(Charge Journal formats data for processing)
Charge Master
(List Price)
DHIS contains a copy of the Charge Master including the "List Price".
Outgoing messages include charge code and account # among other things.
Outgoing message may include price for specific items.
3rd Party Billable
(multiple accounts if needed)
Research Billable
Various Payor rules including agreed upon grant discounts are applied in Transaction Editing System or Billing Accounts Receivable modules
Charge codes with account # and some prices
(multiple accounts if needed)
SAP
General Ledger
Professional Billing to Payors
(including Grants)
Siemens Billing
Contract Manager Module handles Payor pricing rules
RAS
Grant discounts applied
Post Billing Grant Manager Charge Validation
Billing with pricing based on Payor rules
Technical Billing to Payors
(including Grants)
Charge Process Work Flow
Key
Check-in process
Professional Charges
Technical Charges
Billing
Accounting
Post Billing Validation
CTMS Charge Process flow diagram-20060516.ppt
This process depends heavily on the patient being assigned two accounts AND charges being assigned to the correct account.

Why Can't We Leave the Current Structure Alone

- **Industry funded studies**
  - Well financed
  - Tuned to net present value calculation, not to the public health
  - Not required to make results available to public
  - Positive results marketed
  - Need to be rewarded for prioritizing in the public interest

- **NIH/VA/AHRQ**
  - Poorly financed
  - Slow and encumbered by similar peer review system as basic science
  - Informatics islands
  - Difficulty marketing results
  - Need to focus on prioritizing based on impact on the health of Americans

Bureacrats

- Bureaucrats write memoranda both because they appear to be busy when they are writing and because the memos, once written, immediately become proof that they were busy

  Charles Peters

- Hell hath no fury like a bureaucrat scorned

  Milton Friedman (1912 -)

Bureaucracy

- **The only thing that saves us from the bureaucracy is inefficiency. An efficient bureaucracy is the greatest threat to liberty**

  Eugene McCarthy (1916 -), *Time magazine, Feb. 12, 1979*

- **Bureaucracy defends the status quo long past the time when the quo has lost its status**

  Laurence J. Peter (1919 - 1988)

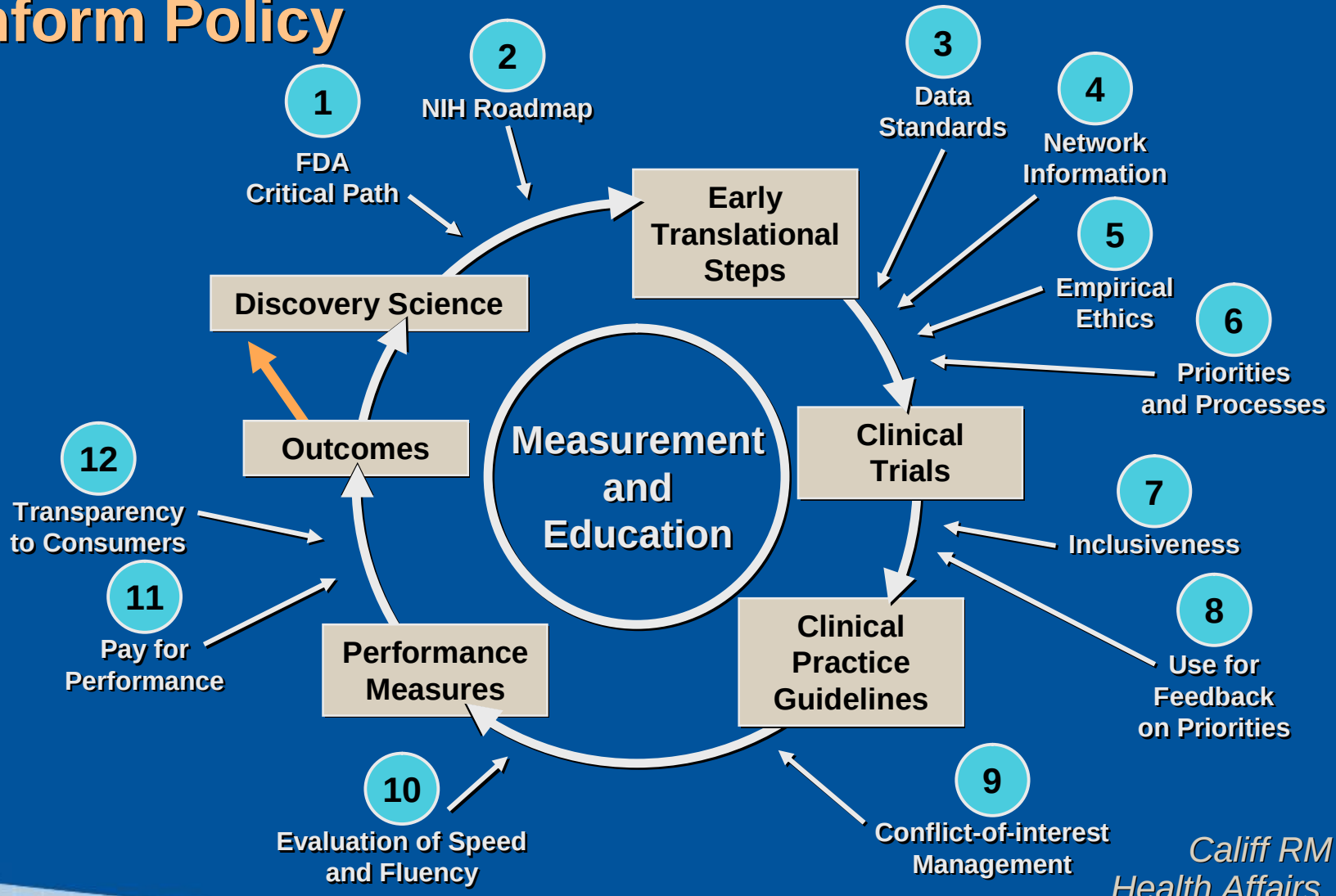
- **Any sufficiently advanced bureaucracy is indistinguishable from molasses.**

  —Unknown

Bureaucracy in Clinical Trials—Good and Bad

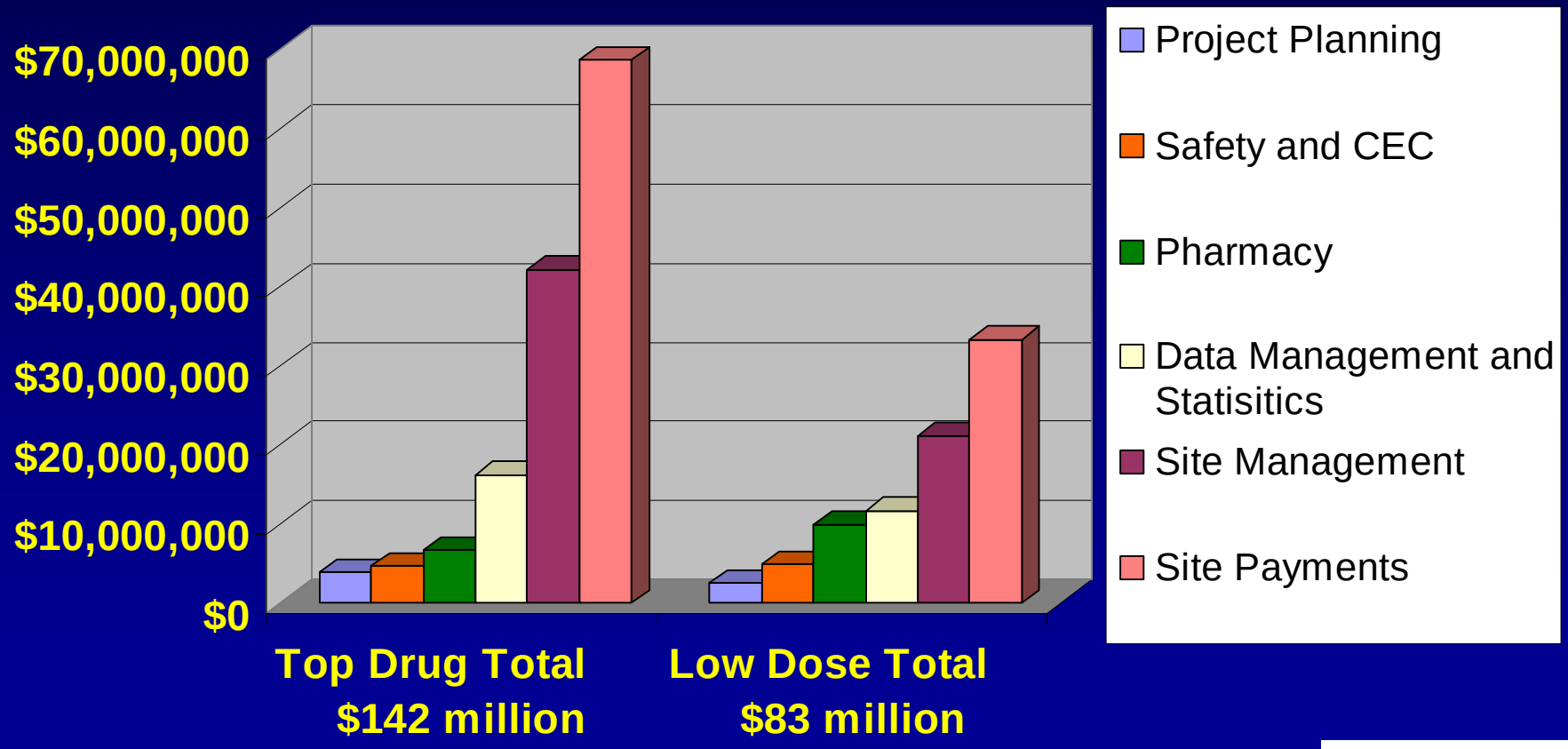
- **Good bureaucracy**
  - Focused on optimizing useful research yield per dollar spent
- **Bad bureaucracy**
  - Armies of people following processes that are not leading to better research answers
  - R&D CEO: “I think of a more efficient way to do something, write a 2 sentence memo, and out comes a 100 page SOP creating an expensive set of procedures to make sure my memo is followed”

The Cycle of Quality: Generating Evidence to Inform Policy



Califf RM et al, Health Affairs, 2007

Estimated Trial Costs



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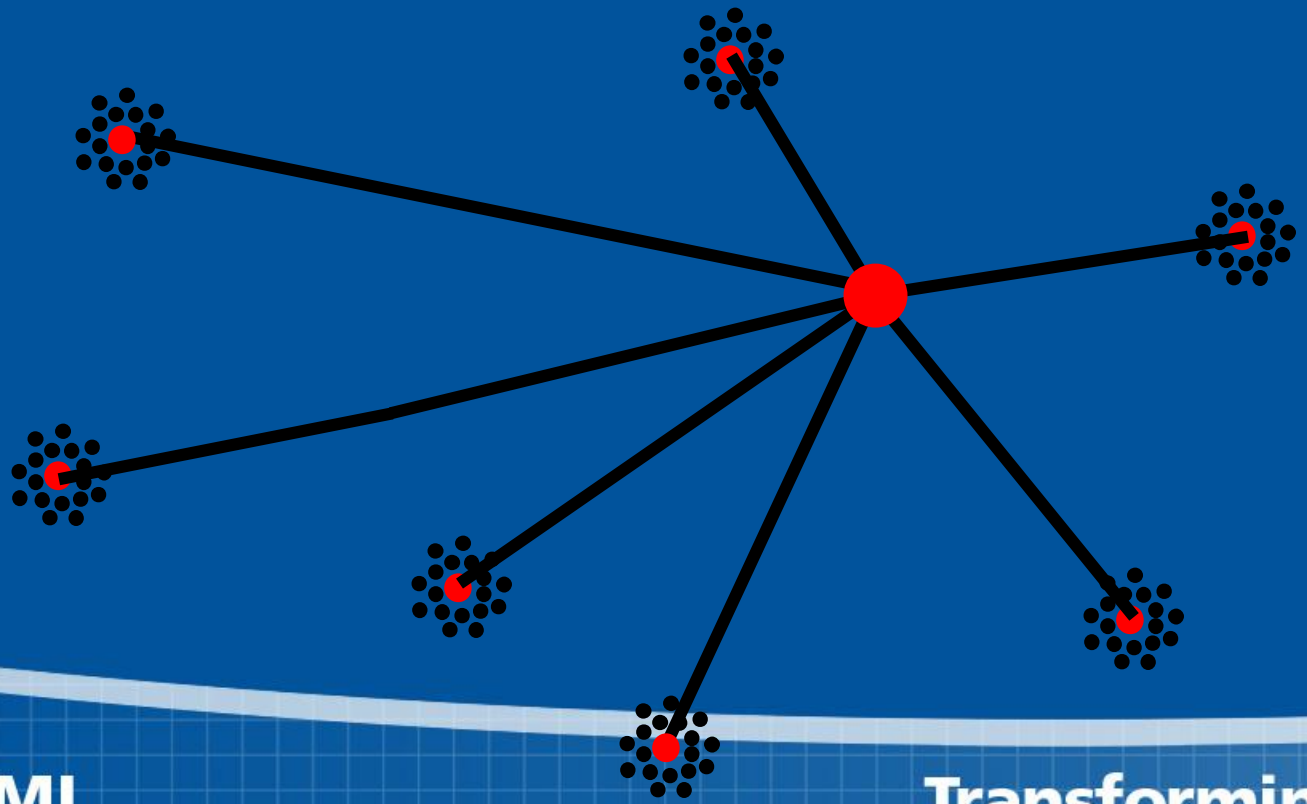
Key Areas for Savings

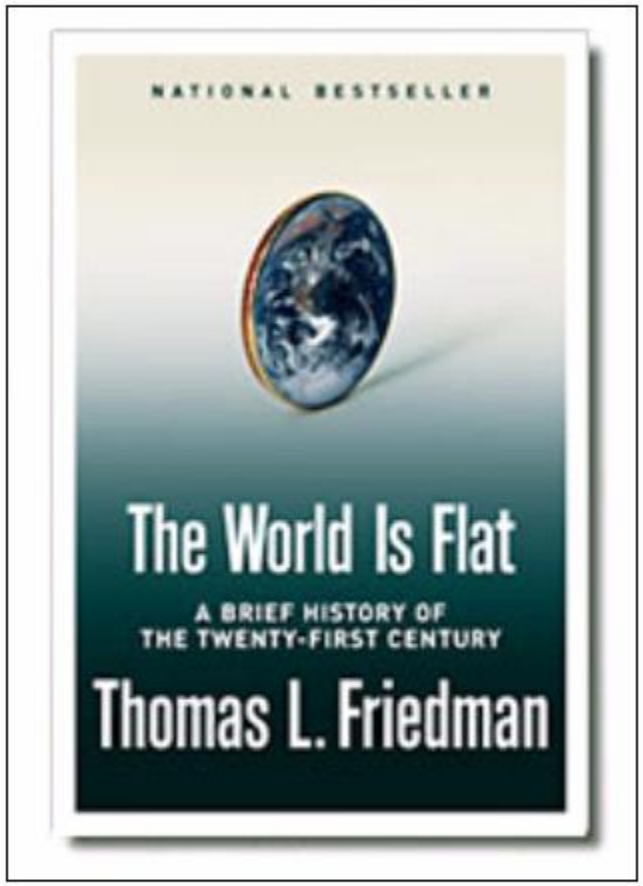
- Simplify protocols
- Data management
- Site monitoring
- Fundamentally a reusable clinical research infrastructure with common standards and nomenclature would cut the cost of clinical research by more than 50%

Typical Clinical Trial: Build it, tear it down, rebuild it, tear it down!



The Challenge of Integration of Clinical Research Networks

- Link existing networks so clinical studies and trials can be conducted more effectively
- Ensure that patients, physicians, and scientists form true "Communities of Research"


NATIONAL BESTSELLER
The World Is Flat
A BRIEF HISTORY OF
THE TWENTY-FIRST CENTURY
Thomas L. Friedman

PATIENT LEAVES HOSPITAL ALIVE

by **Dr James Leftonatrolley**

THERE was wide-spread shock today at the news that a patient had left a hospital today without having been killed by a medical error.

A spokesman for the hospital said, "This is a very rare occurrence and there is no cause for alarm. We will be launching a full enquiry at once into what went right. We can only apologise to the undertakers."