

# Major Barriers to Translating Research into Practice and Policy

Nancy Sung, Ph.D.  
Burroughs Wellcome Fund  
Health Research Alliance

May 18, 2011  
57<sup>th</sup> Meeting of the NCI Director's Consumer Liaison Group  
Chapel Hill, NC

# Outline

- Scope of philanthropic support for medical research
- Barriers/levers
- A few issues/observations

# Funding of US Biomedical Research, 2003-2008

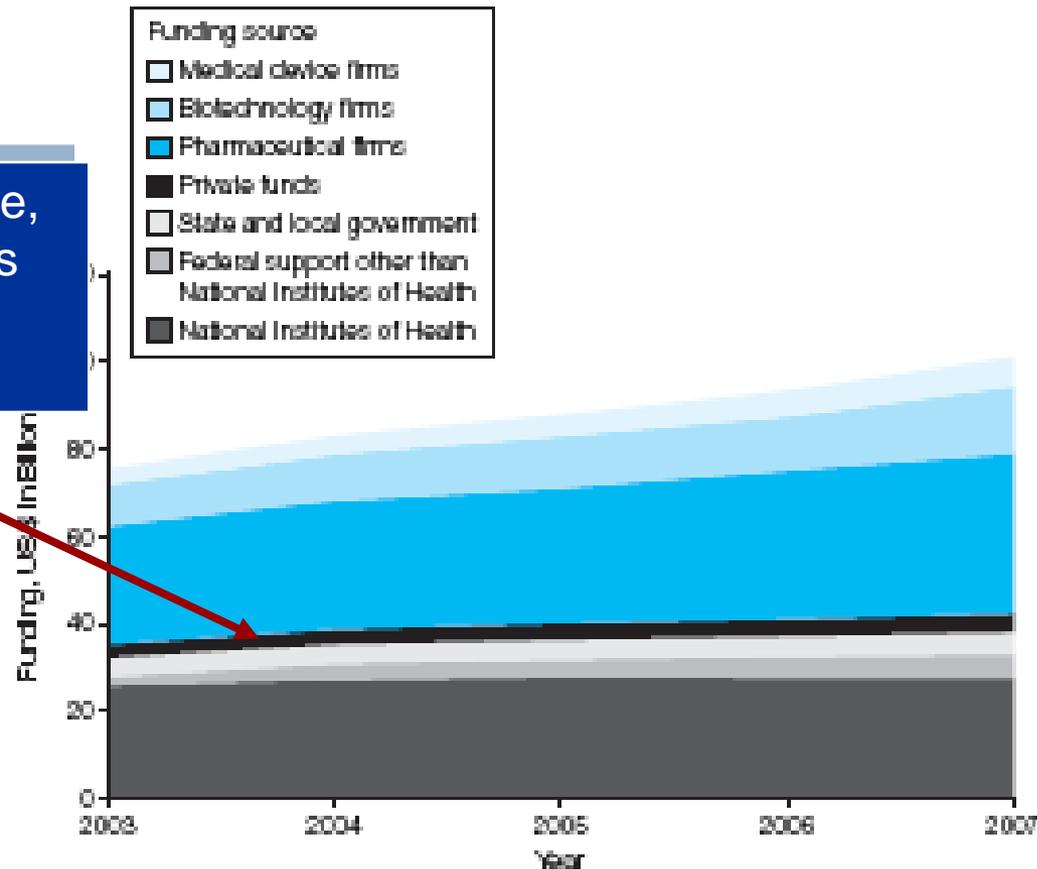
JAMA, January 13, 2010—Vol 303, No. 2

E. Ray Dorsey, MD, MBA  
Jason de Roulet, MD  
Joel P. Thompson, MPH  
Jason I. Reminick, MS  
Ashley Thai, BS  
Zachary White-Stellato  
Christopher A. Beck, PhD  
Benjamin P. George, BS  
Hamilton Moses III, MD

**Context** With the exception of the American Recovery and Reinvestment Act, funding support for biomedical research in the United States has slowed after a decade of doubling. However, the extent and scope of slowing are largely unknown.

**Objective** To quantify funding of biomedical research in the United States from 2003

**Figure 1. Funding for Biomedical Research by Source, 2003-2007**



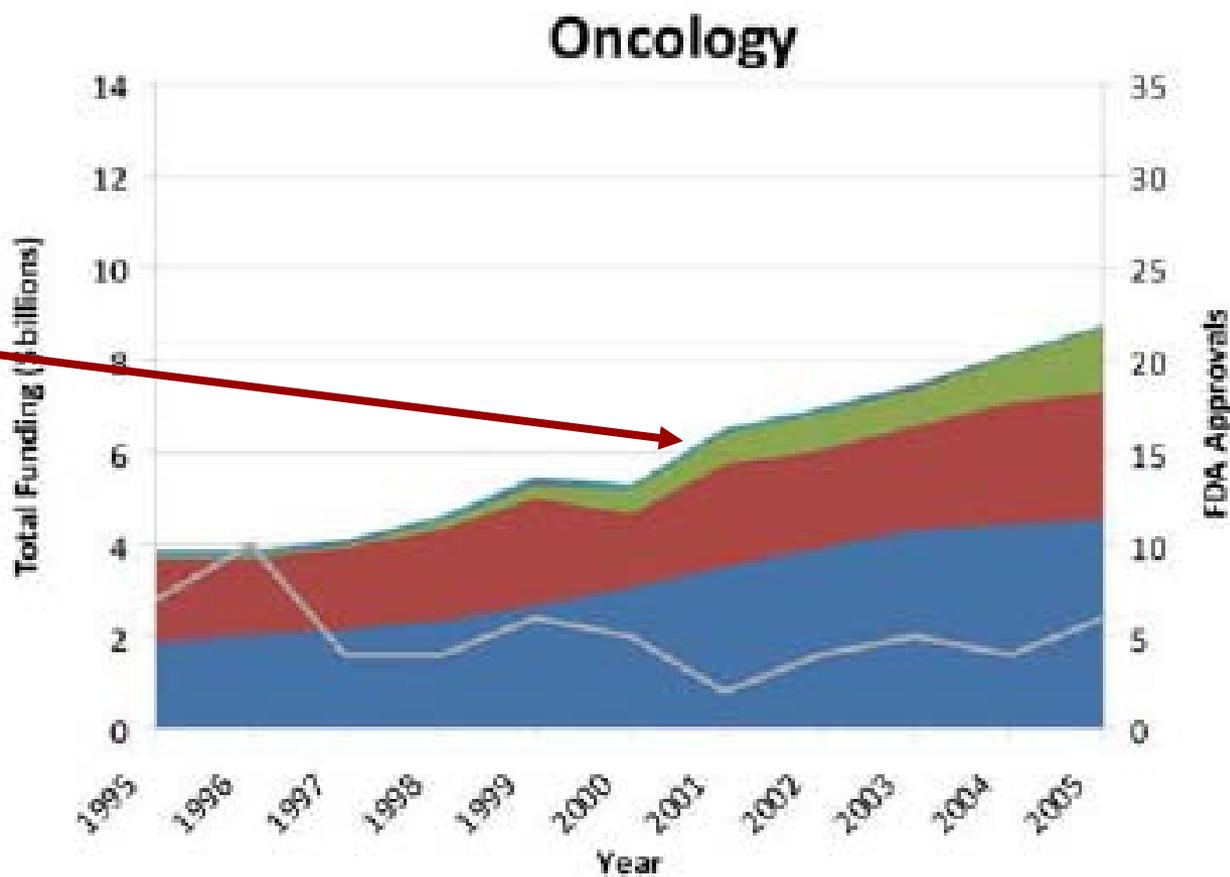
Funding from private, non-profits accounts for ~5% of the enterprise

# Financing of U.S. Biomedical Research and New Drug Approvals across Therapeutic Areas

E. Ray Dorsey<sup>1\*</sup>, Joel P. Thompson<sup>2</sup>, Melisa Carrasco<sup>3</sup>, Jason de Roulet<sup>4</sup>, Philip Vitticore<sup>1</sup>, Sean Nicholson<sup>5</sup>, S. Claiborne Johnston<sup>6</sup>, Robert G. Holloway<sup>1</sup>, Hamilton Moses III<sup>7,8</sup>

<sup>1</sup> Department of Neurology, University of Rochester Medical Center, Rochester, New York, United States of America, <sup>2</sup> School of Medicine & Biomedical Sciences, University at Buffalo, The State University of New York, Buffalo, New York, United States of America, <sup>3</sup> School of Medicine and Dentistry, University of Rochester, Rochester, New York, United States of America, <sup>4</sup> University Hospital Management, Cornell University, Ithaca, New York, United States of America, <sup>5</sup> Department of Neurology, University of Rochester Medical Center, Rochester, New York, United States of America, <sup>6</sup> Department of Neurology, University of Rochester Medical Center, Rochester, New York, United States of America, <sup>7</sup> The Alerion Institute, North Syracuse, New York, United States of America

Based on complete data from only 11 funders

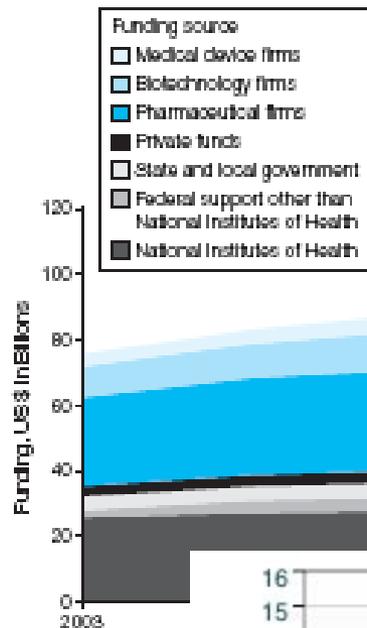


- Not-for-profit Organizations
- Medical Device Firms
- Biotechnology Firms
- Pharmaceutical Firms
- National Institutes of Health
- FDA Approvals

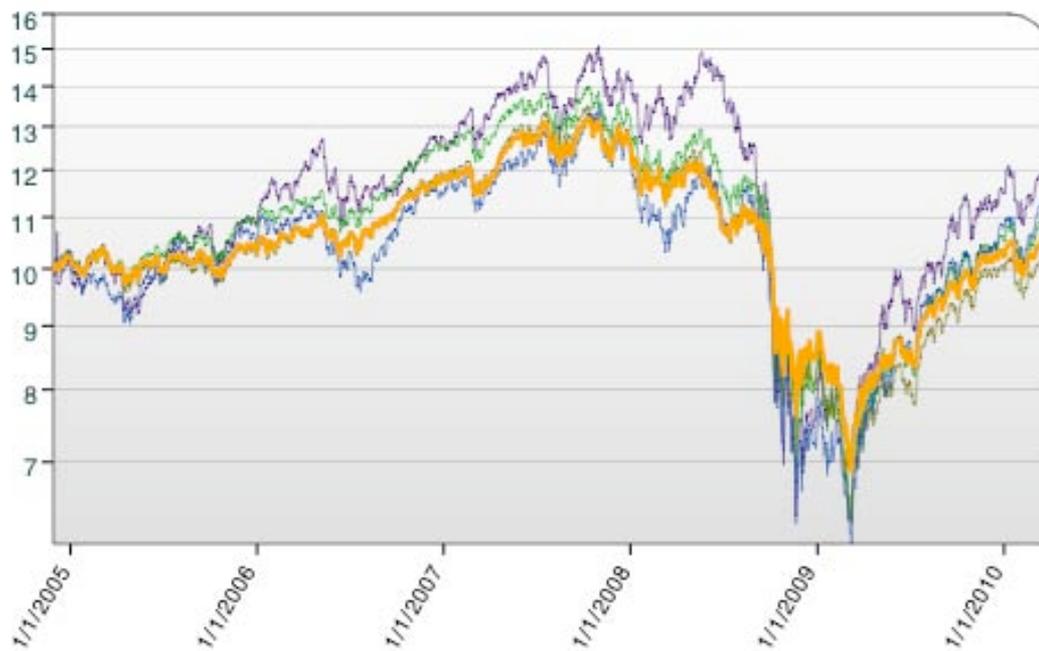
# Funding of US Biomedical Research, 2003-2008

E. Ray Dorsey, MD, MBA  
 Jason de Roulet, MD  
 Joel P. Thompson, MPH  
 Jason I. Reminick, MS  
 Ashley Thai, BS  
 Zachary White-Stellato  
 Christopher A. Beck, PhD  
 Benjamin P. George, BS  
 Hamilton Moses III, MD

**Figure 1. Funding for Biomedical Research by Source, 2003-2007**



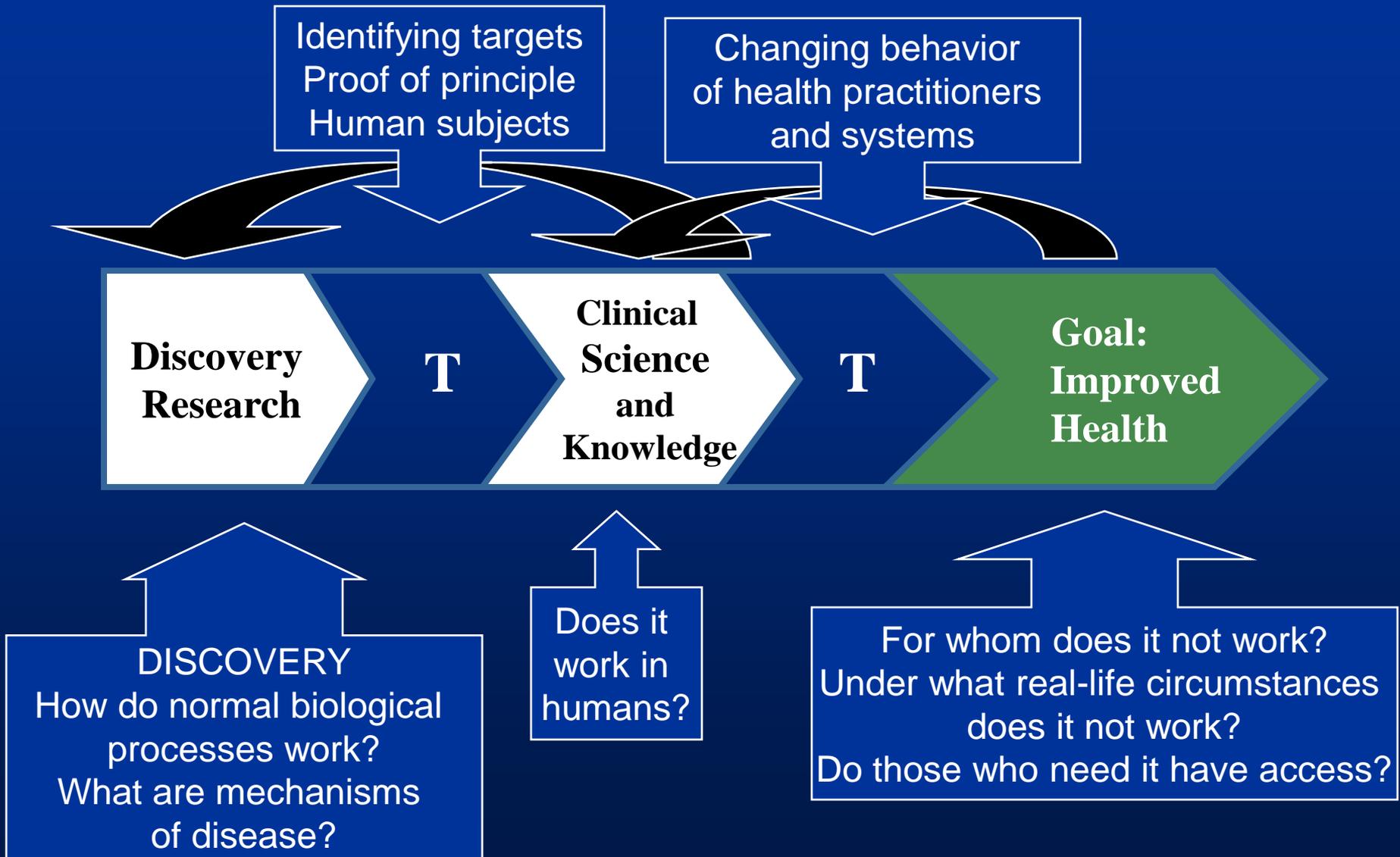
JAMA, January 13, 2010—Vol 303, No. 2



# CATALYST (kăt'l-ĭst) *n.*

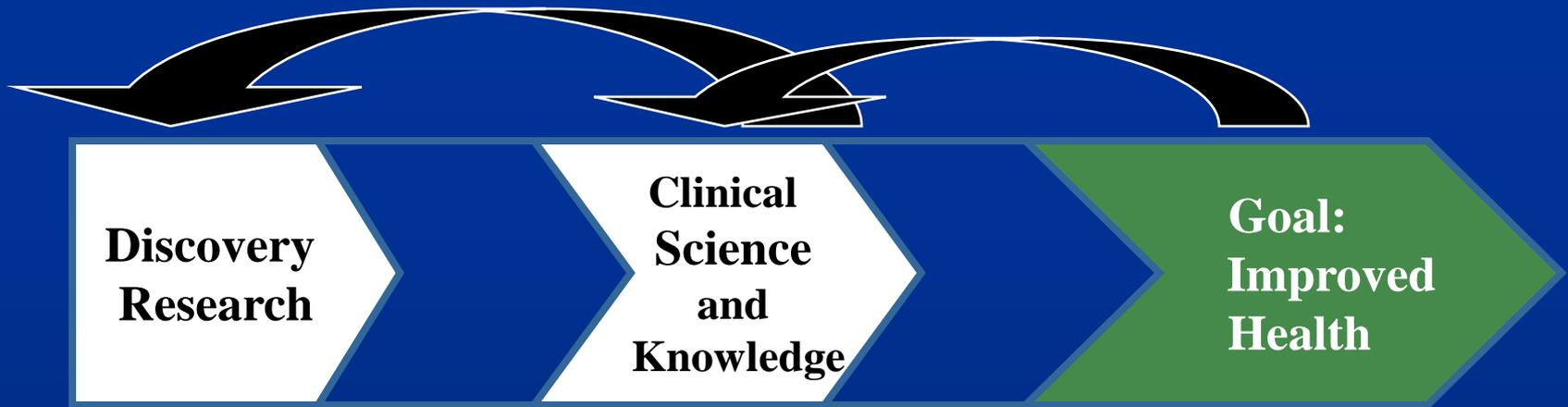
- *Chemistry.* A substance, usually used in small amounts relative to the reactants, that modifies and increases the rate of a reaction without being consumed or changed in the process.

# Biomedical-Health Research Continuum



# Catalytic lever points



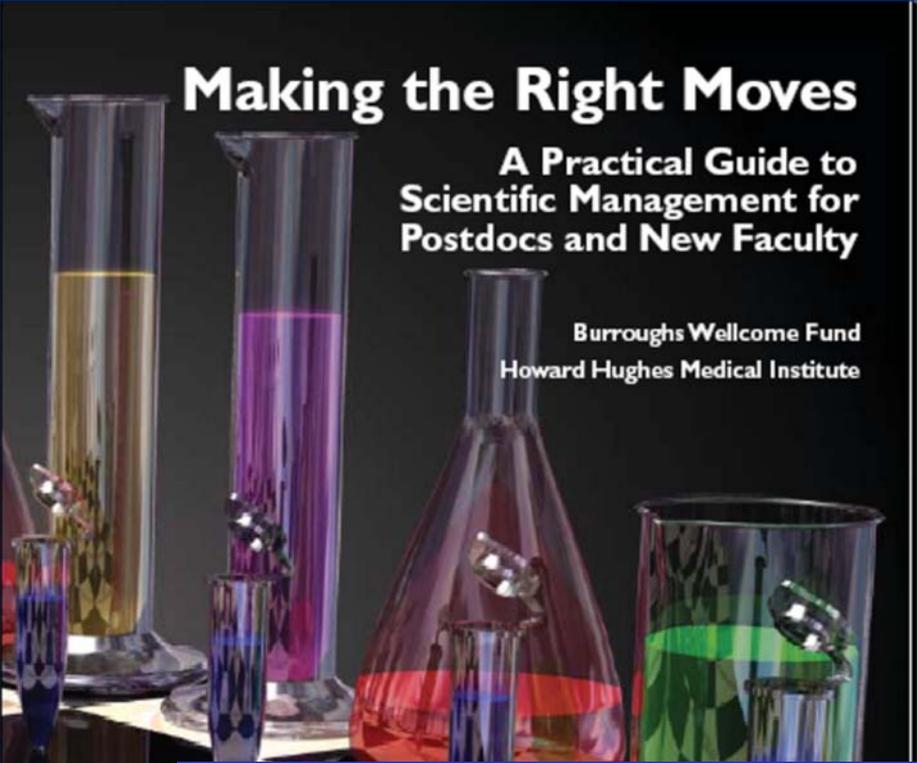


## Barriers to Translating Discovery:

-Career disincentives: harsh payline for grants, uncertainty of success, long training path, debt, academic incentives

-Levers:

Early career funding, debt prevention, training in non-scientific aspects



# Making the Right Moves

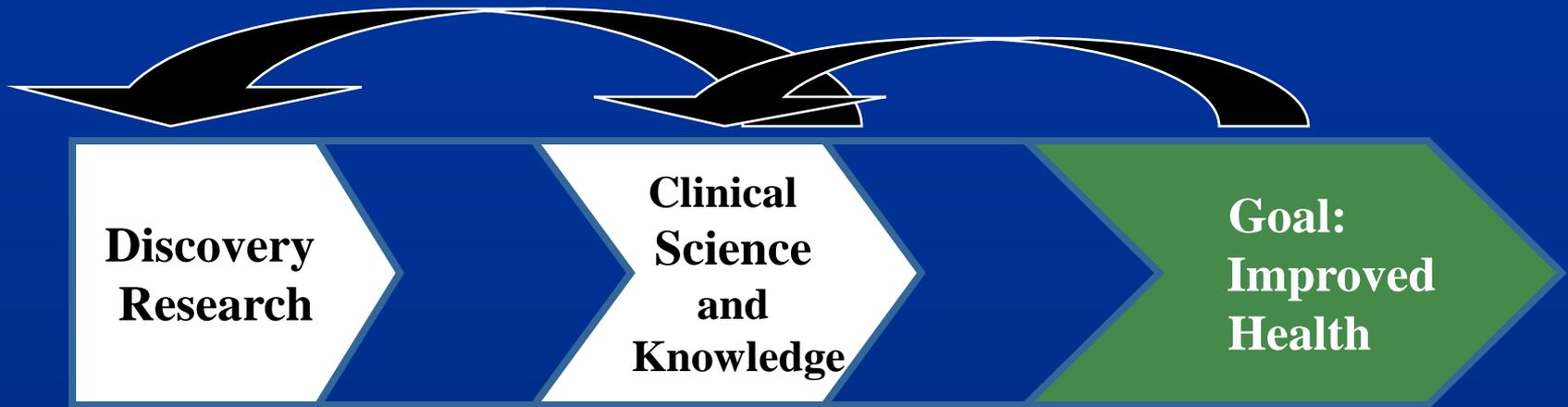
A Practical Guide to  
Scientific Management for  
Postdocs and New Faculty

Burroughs Wellcome Fund  
Howard Hughes Medical Institute

Freely downloadable at  
[www.hhmi.org](http://www.hhmi.org)

2<sup>nd</sup> edition October 2006

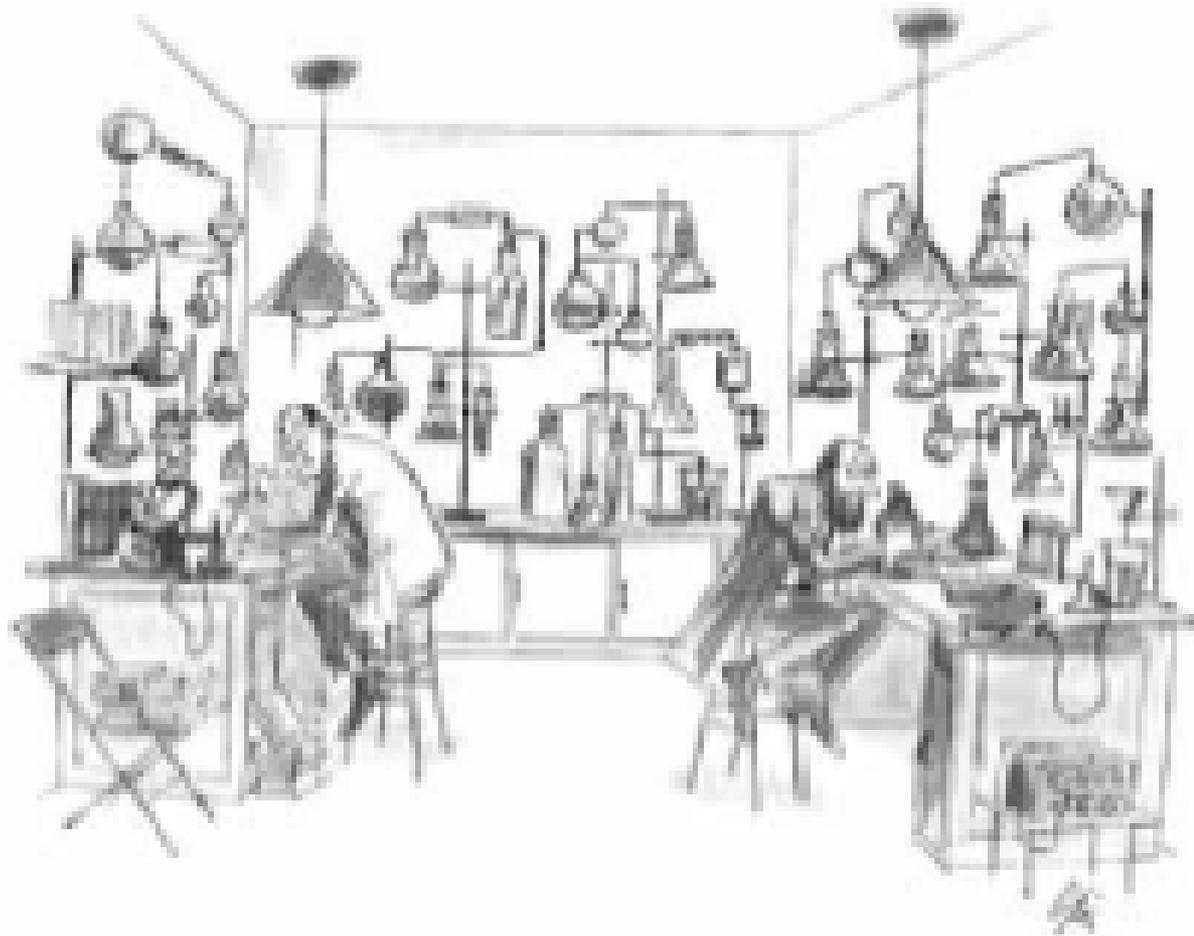
- More than 50,000 postdoctoral appointments in the U.S.
- Tournament model of competition
- An efficient and well-run laboratory requires the skills of a small business owner
- Translated into Chinese



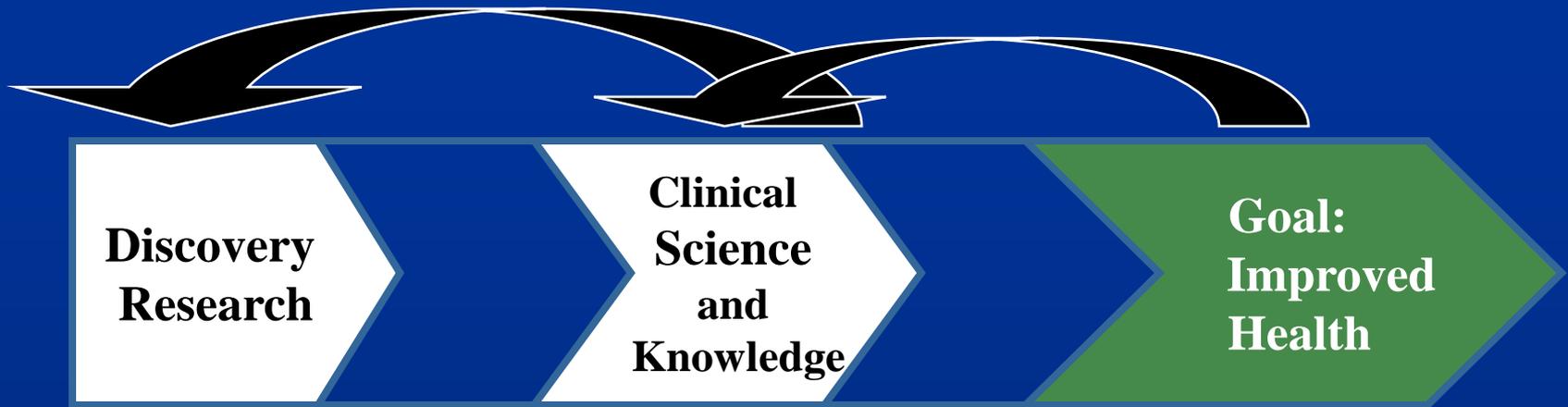
## Barriers to Translating Discovery:

Access to resources (funding, infrastructure, specimens, data)

Academic and industry reluctance to share data



*I see by the current issue of 'Lab News', Ridgeway,  
That you've been working for the last twenty years on the  
same problem I've been working on for the last twenty years."*



## Barriers to Translating Discovery:

Access to resources (funding, infrastructure, specimens, data)

Levers: CCCs, CTSAs, tying funding to sharing, developing standards, benchmarking progress, accelerating knowledge turns

July 11, 2006

## Patients With Rare Diseases Work to Jump-Start Research

### Advocacy Groups Create Their Own Tissue Banks To Aid in Drug Development

By AMY DOCKSER MARCUS  
July 11, 2006; Page D1

Kathy Giusti, 47 years old, was diagnosed a decade ago with multiple myeloma, an incurable and rare cancer of the blood. With only 16,000 new cases diagnosed a year, Ms. Giusti, a former pharmaceutical-company executive, has had a hard time getting drug companies and researchers to study her disease.



Kathy Giusti, founder of the Multiple Myeloma Research Consortium, helped create a biospecimen bank for her disease.

When she asked researchers at a 2004 meeting about the search for treatments, she says they told her they weren't getting tissue from multiple-myeloma patients to study. She decided to give them something to get started. In

Photo: Peter Dinklage

### Foundations Pumping Cash Into Research

#### Venture Philanthropy Is the Model

By HEATHER CHAMBERS  
SAN DIEGO BUSINESS JOURNAL STAFF

Biotechnology firms have typically shied away from developing treatments for rare diseases because the drugs haven't commanded high enough profits.

A drug to treat high cholesterol or depression has the potential to reach more patients than a drug for cystic fibrosis, for instance. The fatal lung condition affects about 30,000 Americans.

But a handful of San Diego firms have succeeded in raising money for less lucrative drug development efforts from nonprofit foundations. San Diego companies such as Amylin Pharmaceuticals, Hollis-Eden Pharmaceuticals, Nervus Pharmaceuticals and Novocell have put the money toward treating so-called "orphan diseases," such as cystic fibrosis, ALS and Huntington's disease.

Biospecimen banks, which collect data about patients

- Funding tissue banks and shared resources in order to break academic silos
- Negotiating for IP/licensing
- Milestone driven
- 'virtual' drug development/PPPs

# Biomedical-Health Research Continuum



## BARRIERS in CLINICAL DEVELOPMENT:

- expense and time
- lack of validated biomarkers
- regulatory science
- clinical trial participation

# LEVERS:

- Absorbing risk
- New trial design, predictive toxicology, modeling: new kinds of scientists are needed
- Creating consortia- ie, for biomarker discovery
- Patient registries, patient education, public understanding

# Examples: reducing the burden of clinical studies:

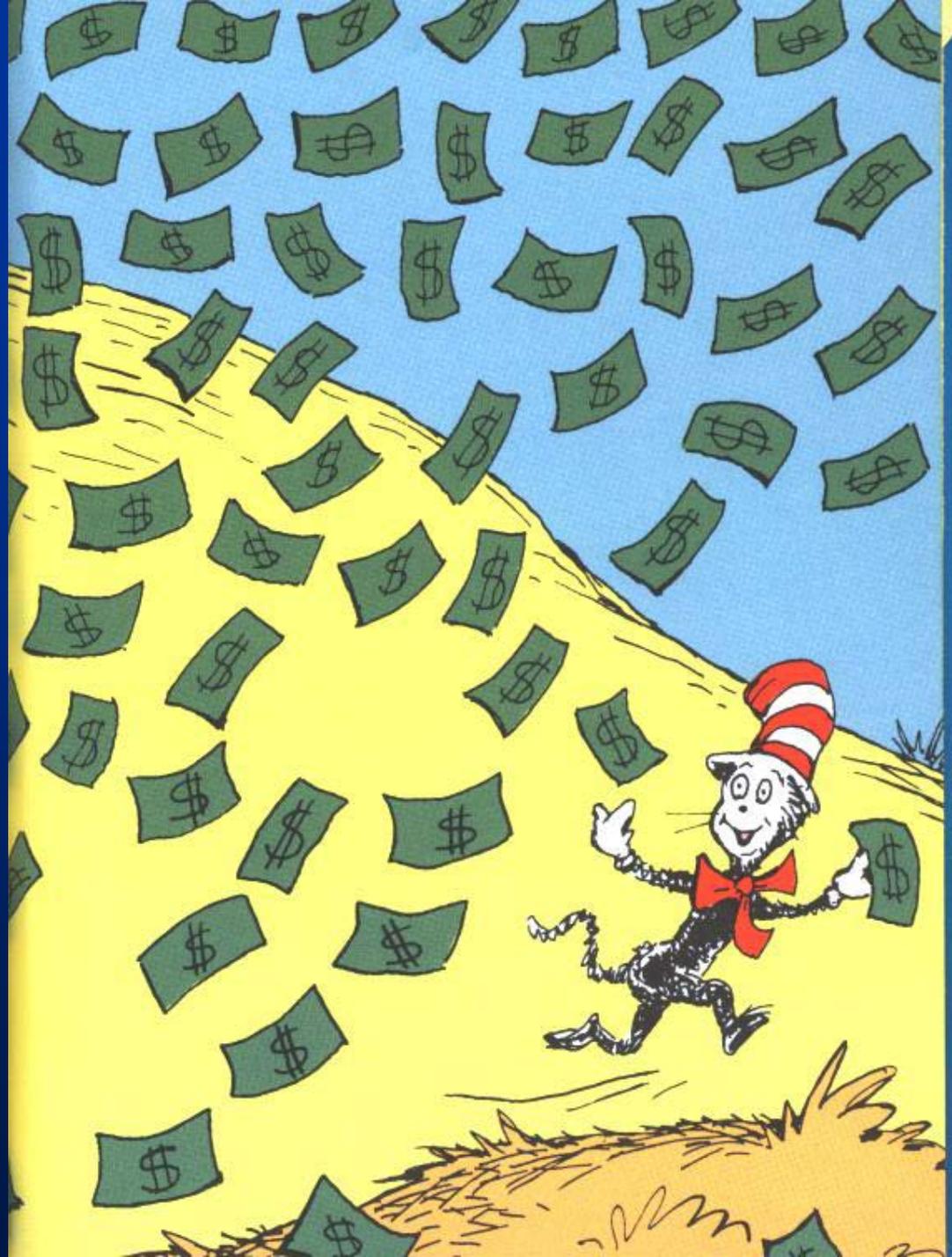
- Muscular Dystrophy Association: supports pre-clinical work necessary for IND application, funds national patient database, supports high-risk early clinical studies, and infrastructure costs
- Foundation Fighting Blindness: Has set up a clinical trial network and preclinical assessment centers which can test compounds at no cost. Planning 3 trials to start in 2010, rapid accrual due to patient registry.

# Some Issues

- Cultural issues: collaboration
- Portfolio approach needed: ie, polio
- Science is at different stages in different fields
- Common vs rare disease
- Public understanding



**It's not just about  
money**





[www.healthra.org](http://www.healthra.org)