Vision for the NIH and the Role of the SMRB

Francis Collins, MD, PhD

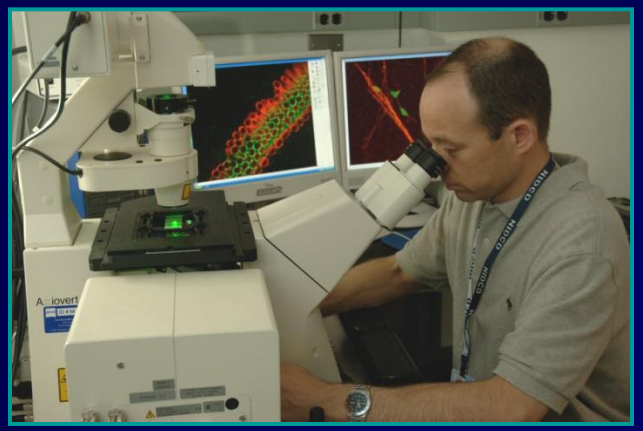
NIH Director

November 13, 2009

My first 94 days as NIH Director…..

NIH: Steward of Medical and Behavioral Research for the Nation

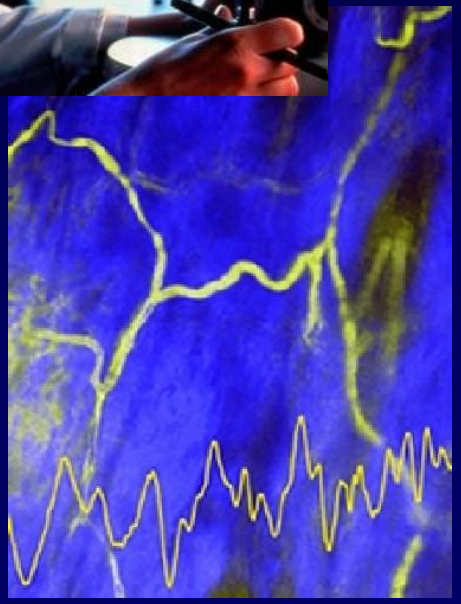
"Science in pursuit of fundamental knowledge about the nature and behavior of living systems... and the application of that knowledge to extend healthy life and reduce the burdens of illness and disability."



Investigator-Initiated Research:
The Foundation of Biomedical Advances

"… the hope of major advances lies in sustaining broad and free-ranging inquiry into all aspects of the phenomena of life, limited only by the criteria of excellence, the scientific importance, and the seriousness and competence of the investigator."

– James A. Shannon, M.D.
8th Director of NIH



Opportunities in Biomedical Research

- **Opportunity #1:**

  Applying unprecedented opportunities in genomics and other high throughput technologies to understand fundamental biology, and to uncover the causes of specific diseases

Opportunities in Biomedical Research

- **NextGen DNA sequencing and beyond**
- **Nanotechnology**
- **Small molecule screening**
- **New imaging modalities**
- **Emphasis on comprehensive approaches**
- **Computational biology is critical**
- **Examples that are ripe for expanded effort**
  - Cancer
  - Autism
  - Microbiome
  - Many more….

Opportunities in Biomedical Research

- **<u>Opportunity #2:</u>**

  Translating basic science discoveries into new and better treatments

- **Moving from…**

Opportunities in Biomedical Research

- To…

Opportunities in Biomedical Research

- **Stage set for NIH to play an 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
- **Small molecules**
  - Roadmap provides HTS capabilities that now exceed that of most pharmaceutical companies
  - Many ICs are ramping up investments
  - Therapeutics for Rare and Neglected Diseases (TRND) program now explicitly tackles the Valley of Death
  - Common diseases are increasingly seen as a collection of rare diseases, so they fit this model too
- **Stem cells (including hESC and iPSC)**
  - Explosion of new information likely with new NIH policy
  - Therapeutic uses uncertain but urgent to pursue

Opportunities in Biomedical Research

- **<u>Opportunity #3</u>:**
  Putting science to work for the benefit of health care reform

Opportunities in Biomedical Research

- **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?

Opportunities in Biomedical Research

- **<u>Opportunity #4</u>:**

  Encouraging a greater focus on global health

Opportunities in Biomedical Research

- **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 long list of neglected tropical diseases (NTDs) affecting hundreds of millions of people worldwide**
- **Include chronic noncommunicable diseases and injuries, responsible for more 50% of deaths in developing world**

Opportunities in Biomedical Research

NIH Roadmap for Medical Research

- **<u>Opportunity #5:</u>**
  Reinvigorating and empowering the biomedical research community

NIH DIRECTOR'S PIONEER A·W·A·R·D

Opportunities in Biomedical Research

- **Strive to avoid the post-ARRA cliff**
- **Emphasis on innovation**
  - Pioneer awards
  - New Innovator awards
  - Transformative RO1s
- **Assessing results of current innovations in peer review**
- **Reinvigorating the Common Fund**
- **Training programs**
  - Seek to reduce age at first grant award
  - Underrepresented minority programs
  - Work force analysis – what's the right answer?

Advisory Committees

- **Four key external committees advise the NIH director:**
  - **Advisory Committee to the NIH Director**
    (NIH Director co-Chairs)
  - **Council of Councils**
  - **Council of Public Representatives**
    (NIH Director co-Chairs)
  - **Scientific Management Review Board**
    (NIH Director is ex officio)

“A committee is a cul-de-sac down which ideas are lured and then quietly strangled.”

Sir Barnett Cocks

(1907 -1989)

“It is better to give than to receive – especially advice.”

Mark Twain
American Author and Lecturer
(1835 – 1910)

Advisory Committee to the NIH Director

- **Focus:**
  - **Program development**
  - **Resource allocation**
  - **NIH administrative regulation and policy**
  - **Other aspects of NIH policy**

- **Specific Activity:**
  - **Advises on grant applications and cooperative agreements for research and training**

Council of Councils

- **Focus:**
  - **Policies and activities of the Division of Program Coordination, Planning, and Strategic Initiatives**
- **Specific Activities:**
  - **Advise on research responsive to emerging scientific opportunities, public health challenges, and knowledge gaps**
  - **Advise the IC Directors during review process for trans-NIH initiatives**

Council of Public Representatives

- **Focus:**
  - **Public participation in NIH activities**
  - **Outreach and public communication efforts**
  - **Other matters of public interest**

- **Specific Function:**
  - **Provides a formal mechanism for public input into the research decision-making and priority-setting process**

Scientific Management Review Board

- **Focus:**
  - **Ensuring the optimal organizational structure and function for the fulfillment of NIH mission**

- **Recommendations are provided to NIH, HHS, and Congress**

Unique Role of the SMRB

- Statutorily-mandated committee to advise on the effective alignment of the Agency structure and function to achieve its goals
- SMRB recommendations will be relevant to the work of the other advisory committees
  - Periodic updates both to and from the ACD, CoC, and COPR
  - SMRB member also serves as ACD member

Key Considerations for the SMRB

- NIH as the "crown jewel" of the Federal government
- Any proposed changes require thorough evaluation and cautious consideration
- Principal driver of change:
  - NIH mission and the Agency's ability to support the best science and address public health needs

Specific Requests to the SMRB

- **Working groups:**
  - **Deliberating Organizational Change and Effectiveness**

    Develop framework for assessing the need for change and develop principles for implementing recommended change

  - **NIH Intramural Research Program**

    Consider changes enabling the IRP to maximize human health and patient well being

  - **Substance Use, Abuse, and Addiction Research**

    Evaluate current organization in light of science and public health

Looking Forward

- **SMRB recommendations that enhance NIH's ability to:**
  - **Maximize scientific discovery and innovation using new research technologies**
  - **Translate research into new diagnostics and therapeutics**
  - **Provide a scientific foundation for health care reform**
  - **Encourage a focus on global health**
  - **Reinvigorate and empower the research community**

Not failure, but low aim, is a crime.

— James Russell Lowell

NIH Scientific Management Review Board

Deliberating Organizational Change and Effectiveness Working Group

Thomas Kelly, MD, PhD
Director
Sloan-Kettering Institute
Memorial Sloan-Kettering Cancer Center

Charge

- **To Articulate:**
  - **The factors and circumstances that might prompt the agency to contemplate organizational change**
  - **A set of principles to guide the consideration of organizational change and its implementation**
- **Always a work-in-progress**
  - **The work of this group will inform, and be informed by, the real-life organizational issues contemplated by the SMRB**

Membership

Non-Federal

William Brody, MD, PhD
(Chair)

Gail Cassell, PhD

Hon. Daniel Goldin

Thomas Kelly, MD, PhD

Eugene Washington, MD

Norman Augustine
(ad hoc)

Federal

Jeremy Berg, PhD

Stephen Katz, MD, PhD

John Niederhuber, MD

Francis Collins, MD, PhD
(ex officio)

Briefings to Date

- **NIH Director's Vision for NIH and the SMRB, including an overview of his 5 opportunities for biomedical research at NIH and reflections upon the group's charge**
- **Perspectives from distinguished scientific and public health leaders on criteria for initiating and implementing organizational change to advancing science and meeting public health needs. Participants included…**

Briefings to Date *(cont...)*

- **National Academy of Sciences Committee: *Enhancing the Vitality of the NIH: Organizational Change to Meet New Challenges***
  - **Kenneth I. Shine, M.D.**
    Executive Vice Chancellor for Health Affairs, University of Texas System
  - **Myrl Weinberg, C.A.E.**
    President, National Health Council
  - **Mary Woolley**
    President, Research!America
  - **Lydia Villa-Komaroff, Ph.D.**
    Chief Scientific Officer, Cytonome/ST
  - **Gilbert S. Omenn, M.D., Ph.D.**
    Professor of Internal Medicine, Human Genetics, and Public Health; Director of the Center for Computational Medicine and Biology, University of Michigan

Perspectives from Panelists

- ***Echoed familiar but nonetheless important themes:***
  - **Increasingly interdisciplinary nature of science**
    - **Need to engage fields beyond the life sciences, including engineering and the physical, informational, and computational sciences and engineering**
    - **Need for new approaches for training next-generation scientists**
  - **Need for increased collaborations**
    - **Within NIH, across agencies, between intra-/extramural, and internationally**
  - **Need for balance between fundamental basic science and translational research**
    - **Importance of basic science as fueling the pipeline of discovery**
    - **Importance of translational research in increasing the impact of NIH on health**
    - **Need for more effective communication with public**
    - **Viewed through the lens of the NIH Director's opportunities in biomedical research**

Principles for Contemplating Change

Contemplated change should:

- **Strengthen the ability of NIH to effectively carry out its mission of advancing science and improving public health**
- **Provide an environment that will enable more effective collaboration, coordination, and interaction across all disciplines to advance the pace of scientific discovery and improve health outcomes**

(still in draft form)

Principles for Contemplating Change *(cont…)*

Contemplated change should: *(cont…)*

- **Bring together units in which there is synergy of the scientific and/or clinical foundation for discovery and translation**
- **Enhance public understanding of, confidence in, and support for science**
- **Increase operational efficiency and ensure a high return on public investment in research**

(still in draft form)

Next Steps

- **Continue to develop draft report, informed by:**
  - **Case studies from government, academia, and industry**
  - **Perspectives from former NIH Directors**
  - **Discussion with experts in organizational change**
- **Circulate draft report to full SMRB**
- **Present draft report for full Board discussion and public comment in March**

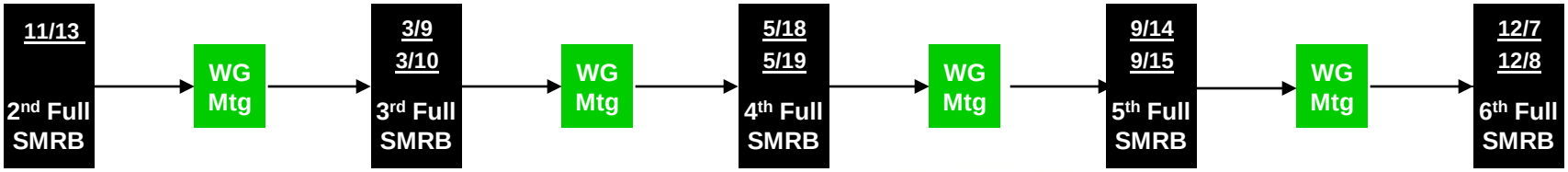
DOCE

Proposed Timeline for DOCE Activities

Seek public input

Present overarching strategies for implementing organizational change at NIH

Publish draft report in Federal Register



Update on briefings and overarching framework

Incorporate stakeholder input

Vote

Present draft criteria and guiding principles

DISCUSSION

NIH Governance and Priority Setting: An Overview

Raynard S. Kington, M.D., Ph.D.
Deputy Director, NIH

Scientific Management Review Board

NIH Reform Act of 2006 Charge to the SMRB

"With respect to a specific contemplated organizational issue:

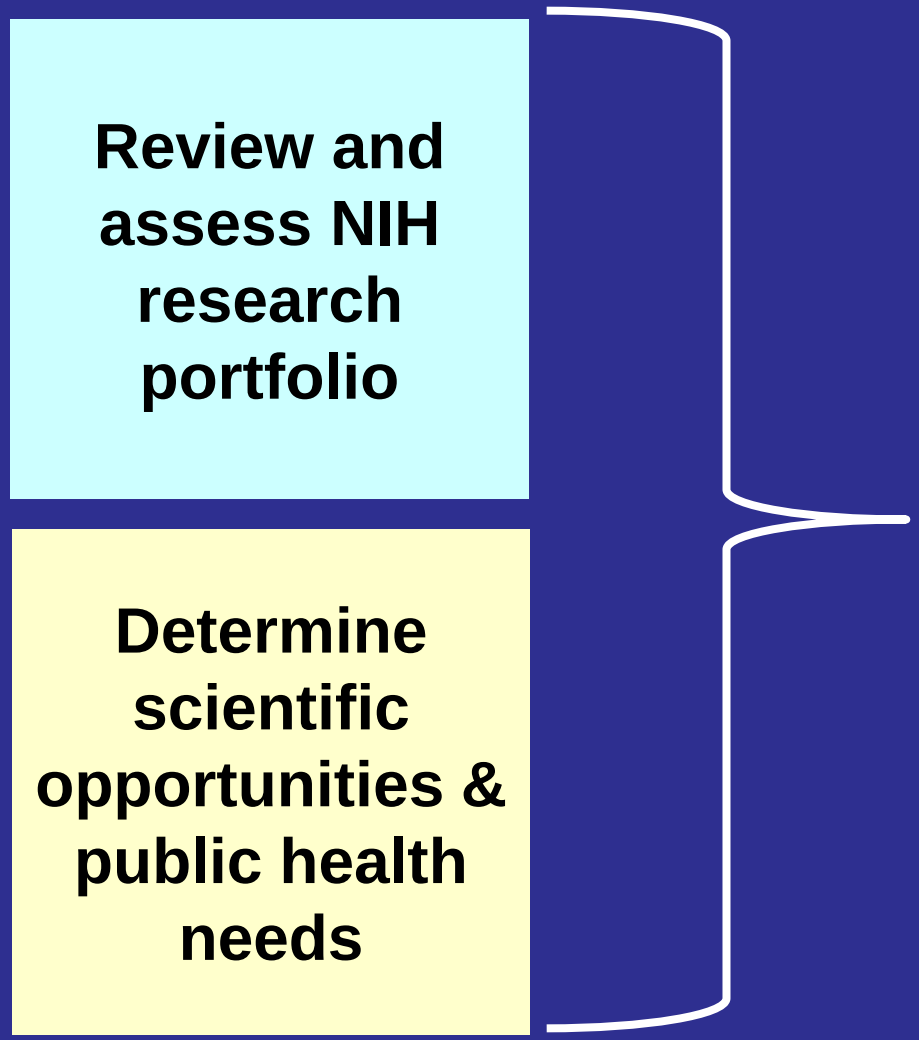
1. Review NIH research portfolio to determine progress, effectiveness, and value of the portfolio and of the allocation of NIH resources among the activities that comprise the portfolio

2. Determine pending scientific opportunities and public health needs with respect to the research within NIH's jurisdiction"

SMRB Charge, continued

3. "For any proposal for organizational change
   - Analyze budgetary and operational consequences
   - Take into account historical funding and support for research activities at national research institutes and centers established recently relative to those in existence for more than two decades
   - Estimate levels of resources needed to implement change(s)
   - Make recommendation for allocating NIH resources among institutes and centers
   - Analyze consequences for the progress of research in areas affected"

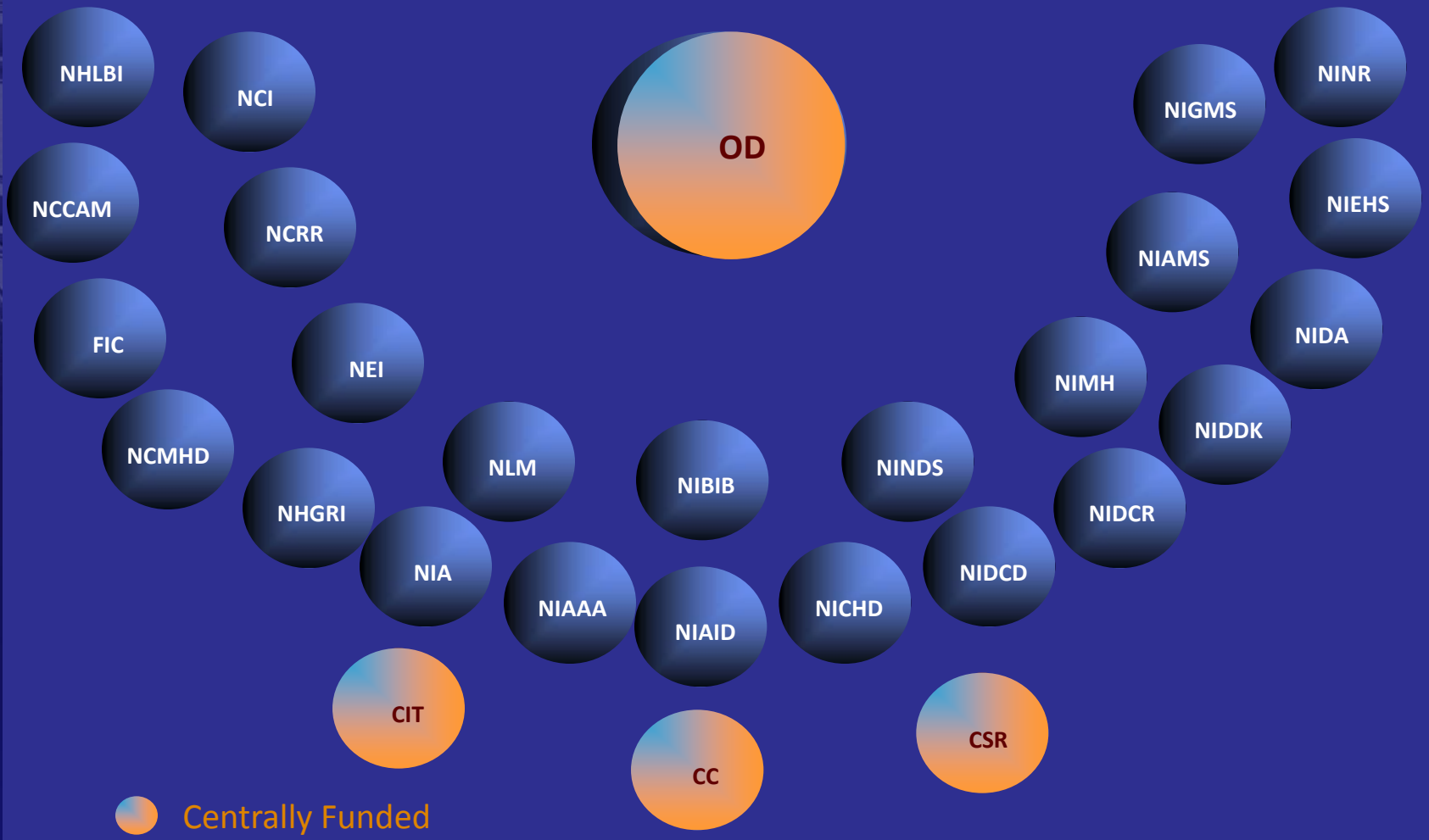
SMRB Charge ➔ This Presentation



Topics

- NIH organizational governance
- NIH priority setting: principles, imperatives, current initiatives and future directions

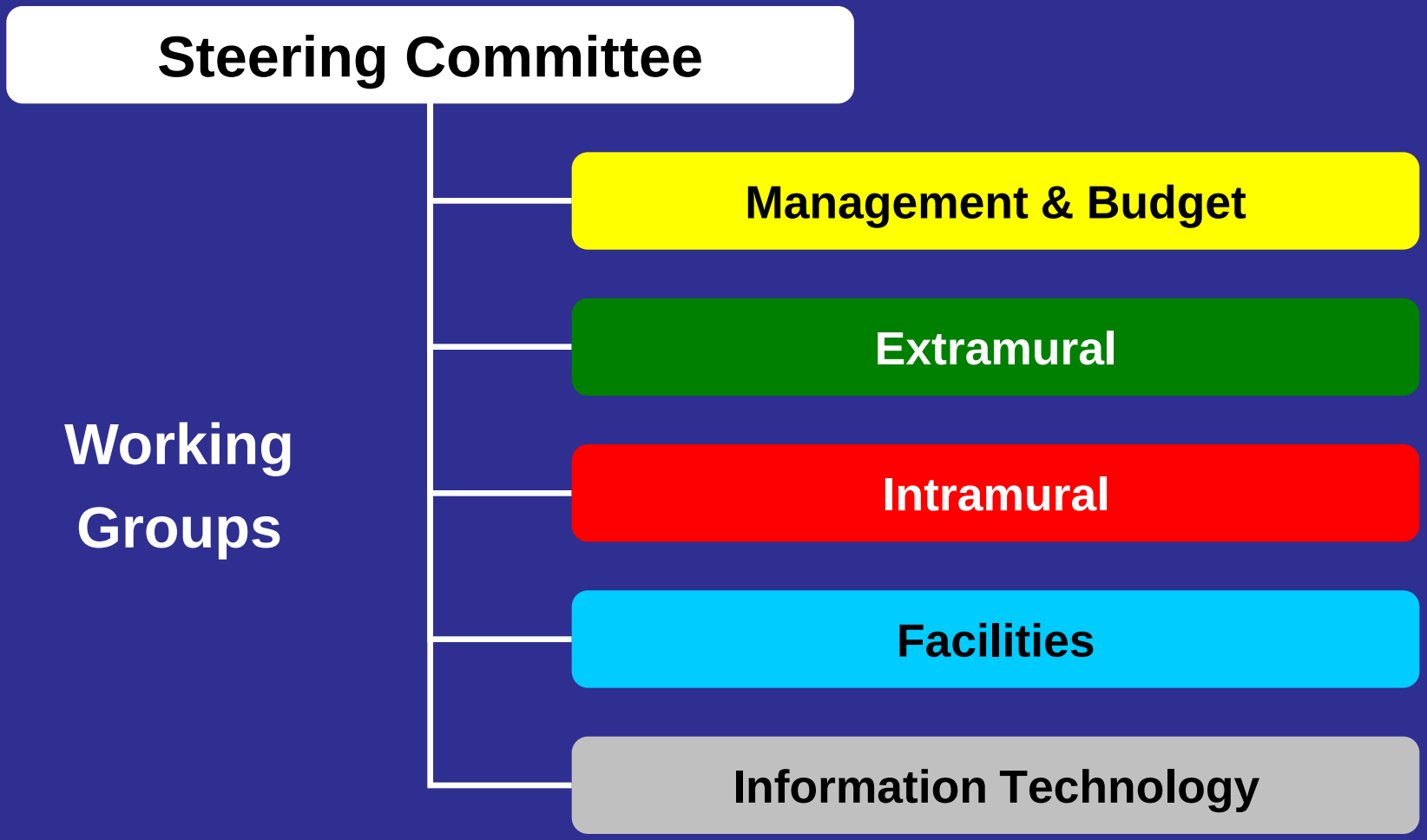
NIH Organizational Structure



Governance: NIH Steering Committee

- Established in July 2004 in response to growth in the size of NIH, complexity of its mission, and the requisite resources
- Has governance purview for all corporate functions, resources, or policies other than the setting of corporate scientific direction and priorities
- Brings operational issues of highest significance to all IC Directors

NIH Organizational Governance



Governance:
Membership of the Steering Committee

- 10 Members
- Chaired by NIH Director
- 3 permanent members: Directors of NCI, NHLBI, and NIAID
- 7 rotating members from other small to mid-size institutes and centers
- NIH Deputy Director is ex-officio member

Governance: Membership of the Working Groups

Each working group is co-chaired by a Steering Committee member and a corresponding senior functional head in the Office of the Director:

1. Management and Budget
2. Extramural
3. Intramural
4. Facilities
5. Information Technology

Governance: Purposes of the Working Groups

- Provide oversight for—but not manage—functions common to ICs
- Make corporate policy and resource recommendations to the Steering Committee
- Issues coordinated by NIH Deputy Director and brought directly to Steering Committee:
  - Science Policy
  - Legislation
  - Communication

Governance: Review and Revision

- Two reviews completed to date
- Conclusions of internal evaluation of Working Groups:
  - Overall governance structure has improved the decision-making process
  - Potential areas for improvement:
    - Unclear role of Legacy Committees, particularly Scientific Directors
    - Enhancing coordination among working groups for cross cutting issues
    - Unclear scope of “authority” for Working Groups
- NIH Director currently reviewing governance

Background on NIH Research Priority Setting

- Focus on extramural research
- Peer review to drive decision-making
- Importance of investigator-initiated research: the "Invisible Hand" in the free market of scientific ideas
- Congress defers to the rigorous process driven by the scientific community
  - *"… the day we decide which grants are going to be approved… is the day we will ruin science research in this country. We have no business making political judgments about those kinds of issues."*

~ Representative David Obey
House Appropriations Committee Chairman
July 11, 2003

Priority Setting: Current Processes

Multi-leveled, multi-focal

Priority setting occurs within every institute/center

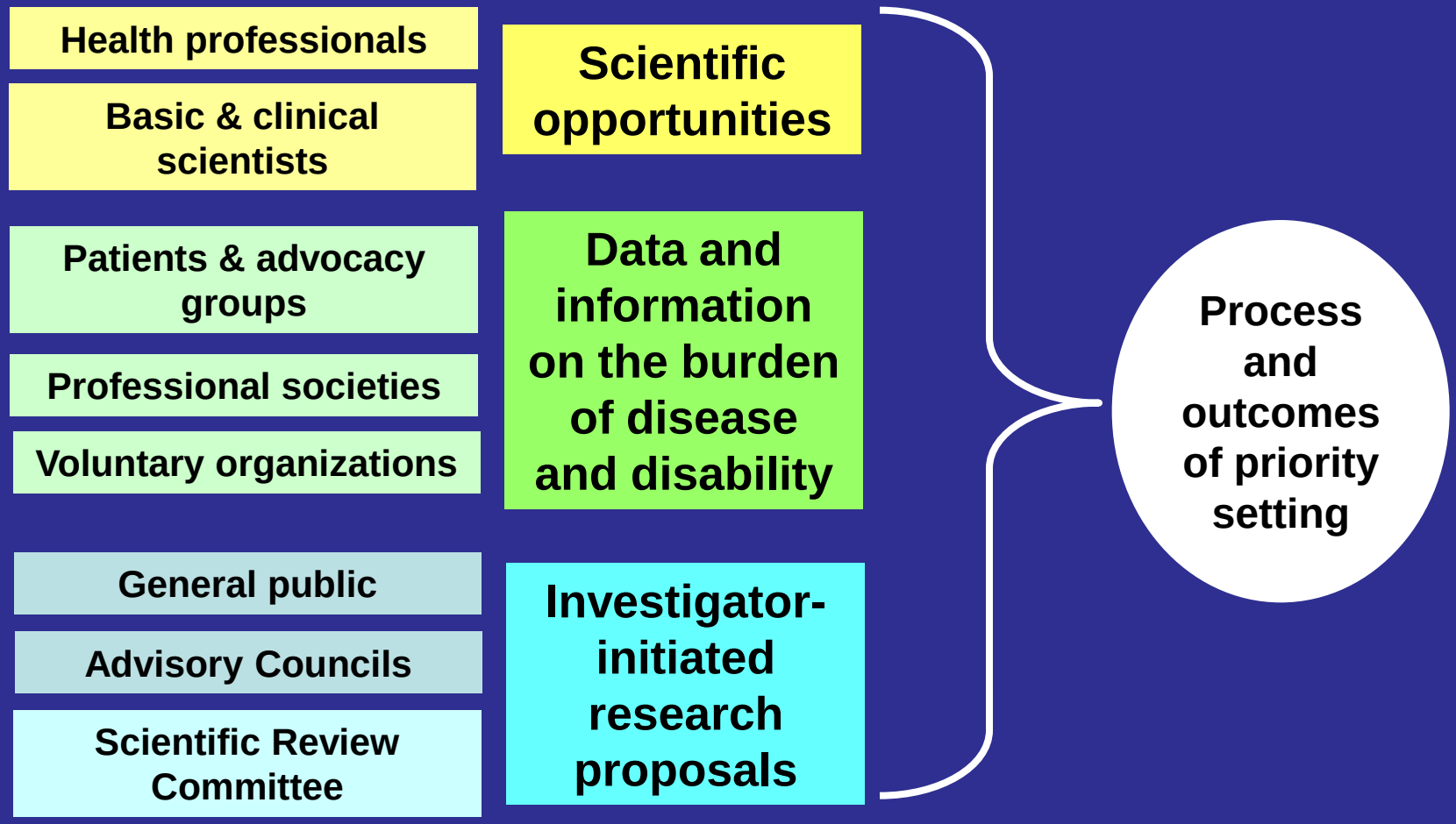
Priority setting occurs within the Office of the Director

Priority setting occurs beyond NIH in the executive and legislative branches of the federal government

Priorities reflected in resource allocations, especially budget

Priority Setting: Current Processes

Multiple "inputs": stakeholders, data, and information



Setting Research Priorities – Many Stakeholders

General Public

Boards of Scientific Counselors

Patients & Their Advocacy Groups

Voluntary Organizations

NIH Staff

Scientific Review Committees

Congress

Scientist Council Members

U.S. President

Scientists

Ad Hoc Advisors

Public Members of Advisory Councils

Physicians & Other Health Professionals

Professional Societies

Foreign Governments

Industry

Priority Setting: Current Processes

… complex and multifaceted …

"Some of the variables in choosing resource allocations include public health needs such as the burden of disease, new scientific opportunities, the quality of research proposals, the experience of applicants, and the ability to sustain research through adequate staffing and infrastructure..."

~ Dr. Elias Zerhouni
Testimony before the Committee on Energy and Commerce, Subcommittee on Health, United States House of Representatives
March 17, 2005

Priority Setting: Current Processes
Executive and Legislative Branches

- U.S. Congress
  - NIH authorization and appropriations
  - Sets NIH and IC funding levels and directs NIH attention to particular areas of research interest or emphasis
  - Historically influential in organizational change at NIH, e.g., through creation of new ICs
- Executive Branch, White House, OMB, HHS
  - NIH appropriations process
  - Establishes priorities for improving health, e.g. Healthy People 2010

Priority Setting: Current Processes
Institutes and Centers

- Primary locus of research priority setting
- Priorities partially driven by scientific community through investigator-initiated research applications
- Two-tiered peer review process:
  - Assessment of scientific and technical merit
  - Review by Advisory Councils

Priority Setting: Current Processes
IC Advisory Councils

- Comprised of senior scientific experts and members of the public
- Advise and recommend programmatic relevance of applications and areas of science to emphasize to the ICs
- Vet ideas for scientific initiatives that receive set-aside funds
- Advise ICs on scientific priorities

Priority Setting: Current Processes
Trans-NIH Planning

- Creation of the Division of Program Coordination, Planning and Strategic Initiatives (DPCPSI) in 2007
  - In the Office of the NIH Director
  - Created to identify important areas of emerging scientific opportunity, rising public health challenge, and gaps in knowledge that deserve special emphasis
  - Home of the NIH Roadmap and Common Fund

Priority Setting: Current Processes

Trans-NIH Planning – Roadmap and Common Fund

- Programs are developed via highly dynamic strategic planning process
- Provides NIH with flexibility to respond quickly to new ideas, challenges, and gaps
- Involves broad stakeholder input from multiple scientific and public sources as well as NIH leadership

Priority Setting: Current Processes
Trans-NIH Planning – Roadmap and Common Fund

- NIH solicited ideas for new initiatives from intramural and extramural scientific community, health professionals, patient advocates, and general public
  - (RFI in NIH Guide: http://grants.nih.gov/grants/guide/notice-files/NOT-RM-08-014.html)
- Programmatic review of submitted ideas, assessment in light of current NIH portfolio, vetting by Council of Councils, and final review by NIH leadership

Priority Setting: Current Processes

Trans-NIH Planning – Roadmap and Common Fund

Proposed initiatives must:

1. Be truly transforming
2. Promote and advance the individual missions of the Institutes and Centers (IC) to benefit health
3. Require participation from NIH as a whole and/or address an area(s) of science that does not clearly fall within the mission of any one IC or program office
4. Be unique

Priority Setting: Current Processes Trans-NIH Planning – DPCPSI

- Coordinate development of tools to enhance portfolio analysis and priority setting
  - Create better information systems to analyze the entire NIH portfolio of research and provide modern decision support systems to all Institutes and Centers
    - Research, Condition, and Disease Categorization (RCDC) system
    - Portfolio Analysis Pilot Projects
    - Institute and Center-developed efforts, e.g., NIAID electronic Scientific Portfolio Assistant (eSPA)

Priority Setting: Current Processes Trans-NIH Planning – DPCPSI

- Enhance efforts to report on outputs, outcomes, and return on investment
  - Research Portfolio Online Reporting Tool (RePORT)
  - Expand measures of Scientific Opportunity vs. Public Health burden and societal demands within each IC and across NIH

Priority Setting: Current Processes

"Some of the variables in choosing resource allocations include public health needs such as the burden of disease, new scientific opportunities, the quality of research proposals, the experience of applicants, and the ability to sustain research through adequate staffing and infrastructure. These factors are often lost in the public debate about NIH funding, in which the discussion is sometimes simplified by focusing attention on apparent differences between the toll of certain diseases and the amount spent on research about those diseases."

~ Dr. Elias Zerhouni

Testimony before the Committee on Energy and Commerce, Subcommittee on Health, United States House of Representatives
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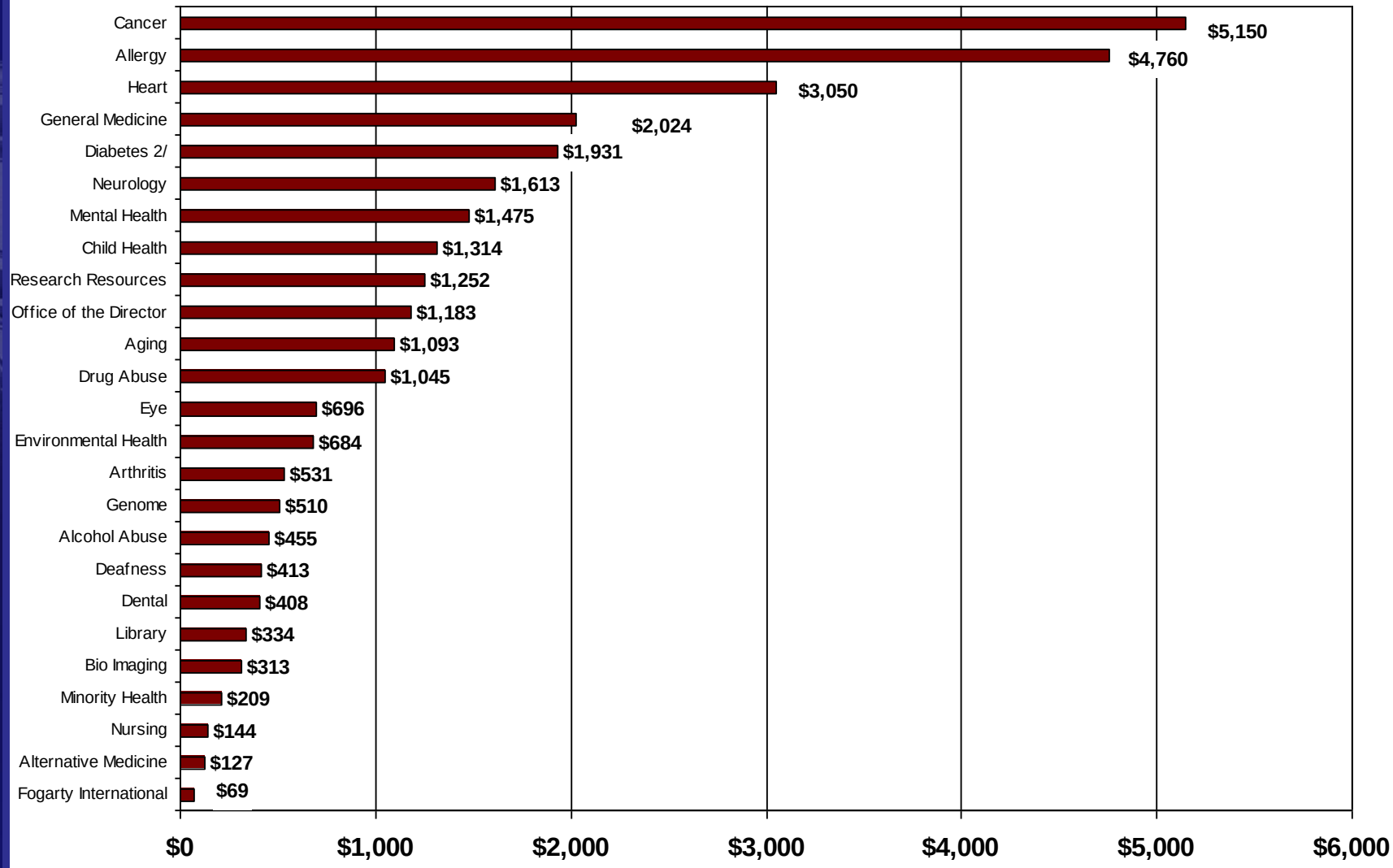
Budgets and Funding as Reflections of NIH's Priorities: Differing Perspectives

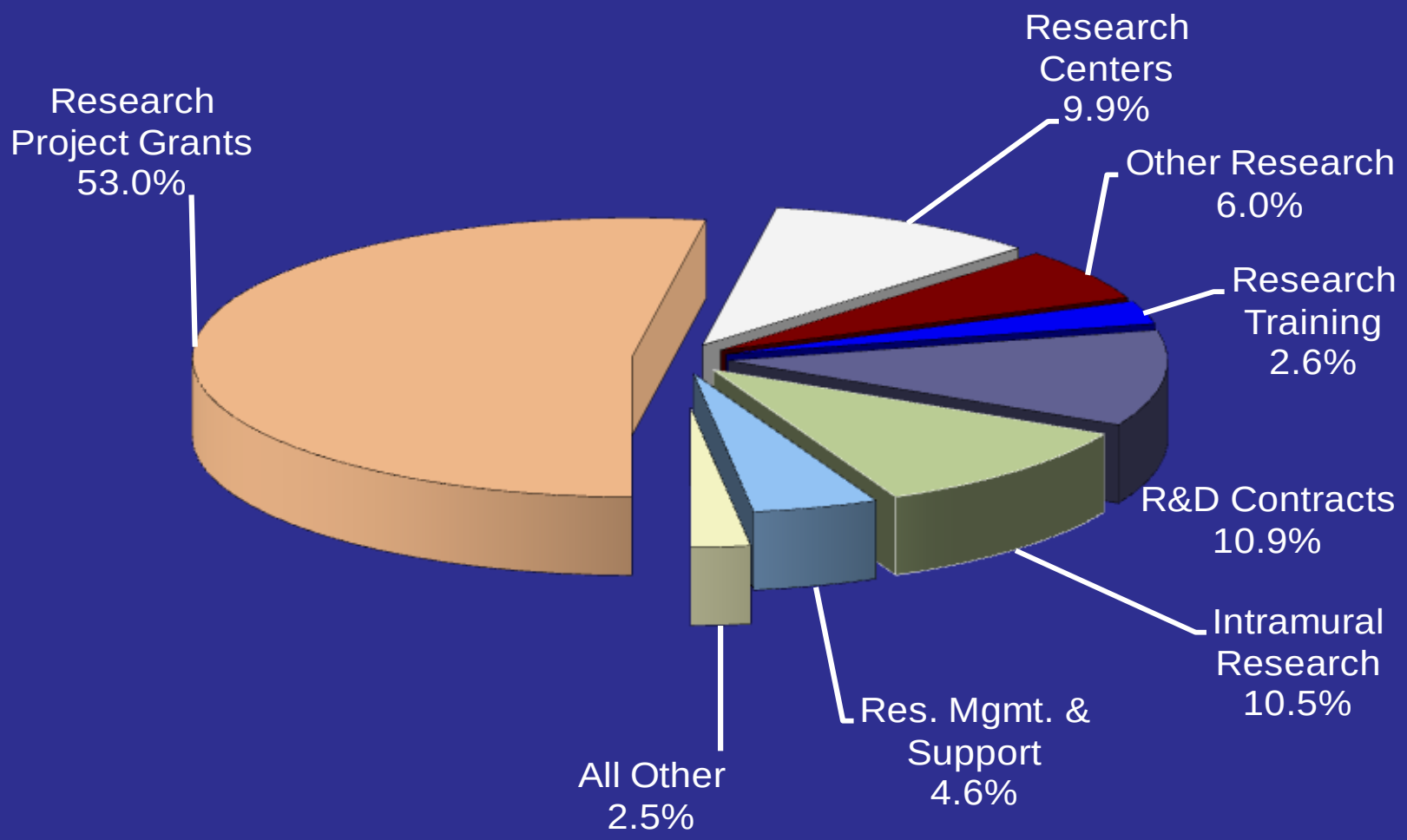
- By Institute, Center, Office
- By Mechanism
- By Spending at and to Entities Outside NIH
- By Research/Disease Areas
- By Likelihood of Application Being Funded
- By Disease Burden

FY 2010 President's Budget NIH Funding By Institute/Center[1]
($ in Millions)

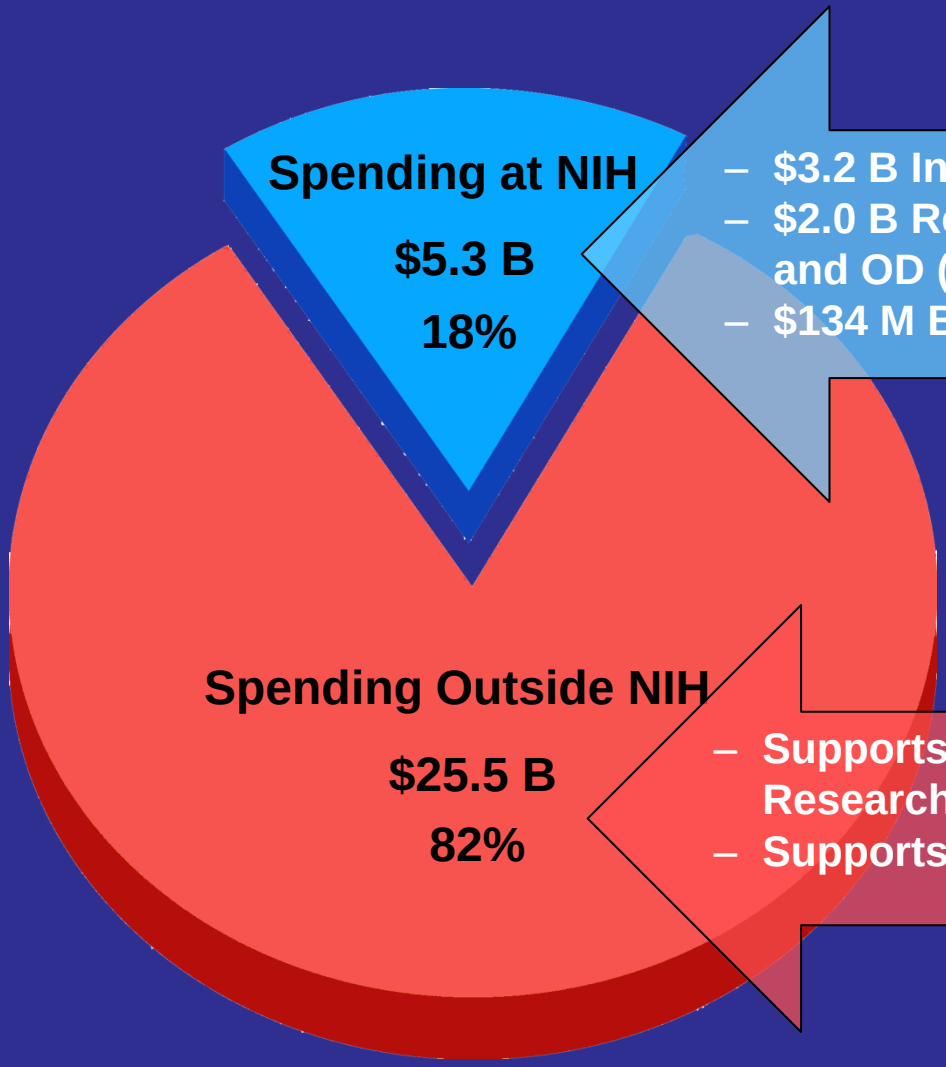


1. Includes funding for Type 1 Diabetes.

FY 2010 President's Budget Request
NIH Budget Authority $30,759M



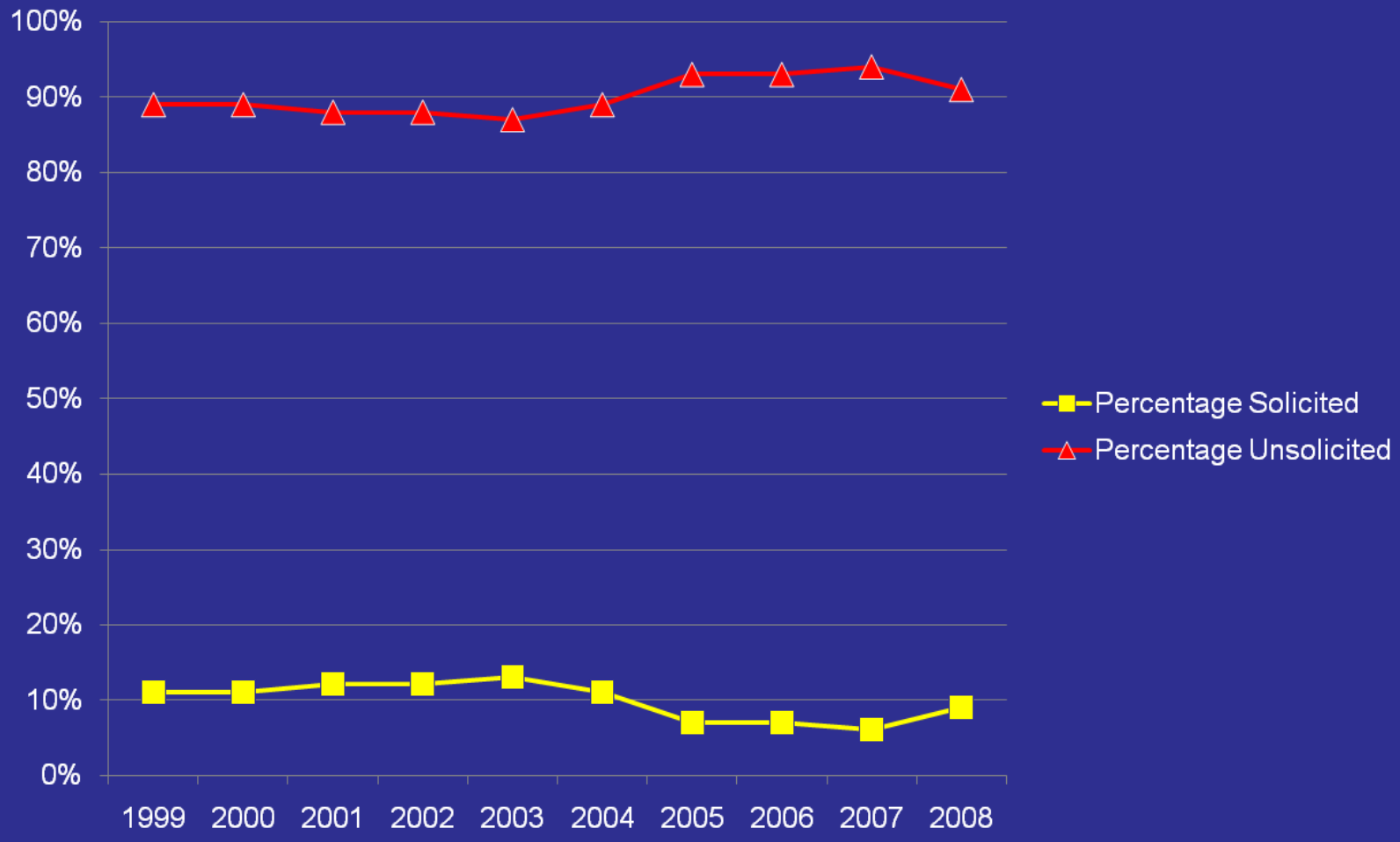
NIH Funding by Mechanism
FY 2010: $30.759 B



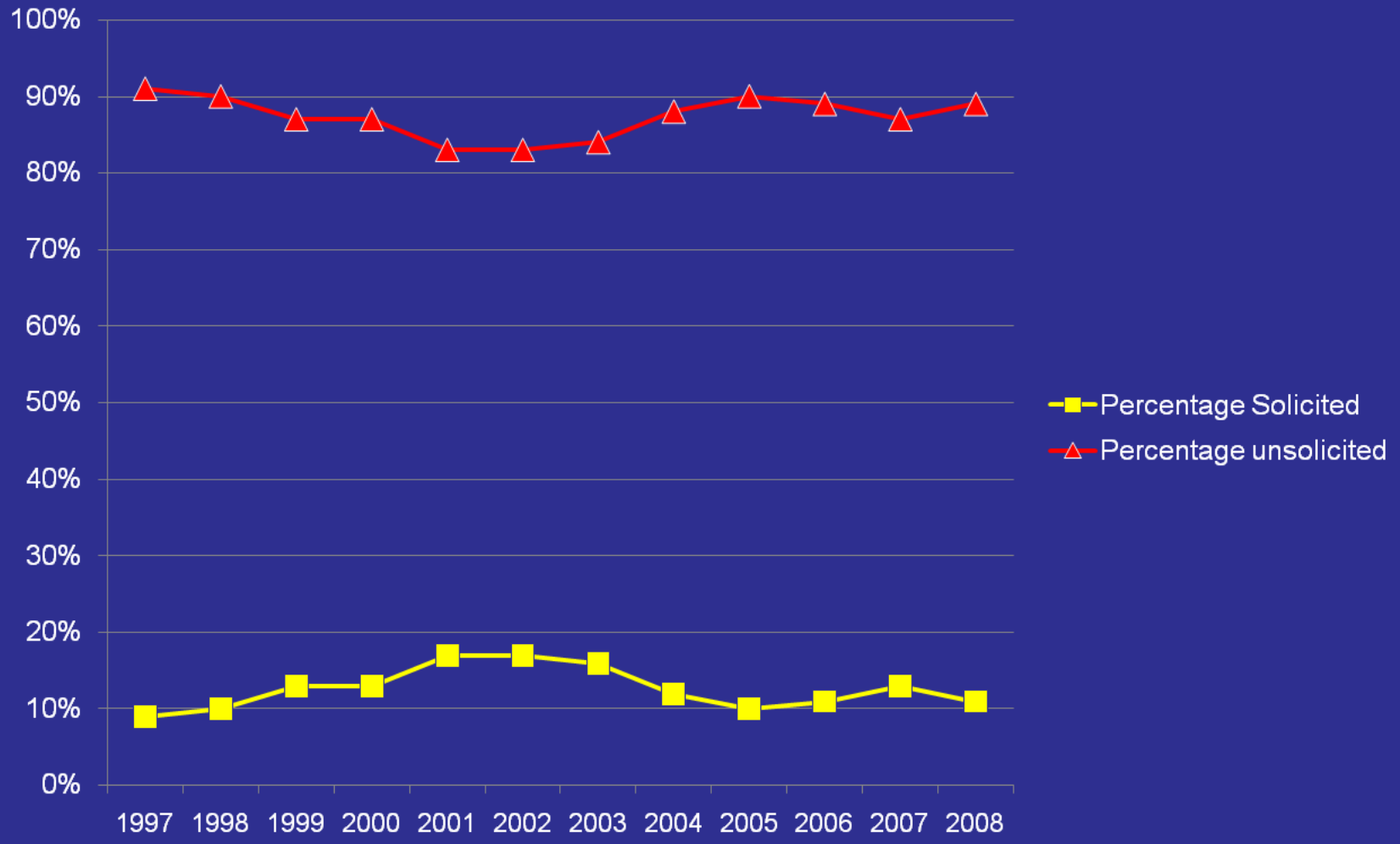
- $3.2 B Intramural Research = 10.4%
- $2.0 B Research Management & Support and OD (w/o Common Fund) = 6.7%
- $134 M B&F = 0.4%

- Supports over 325,000 Scientists & Research Personnel
- Supports over 3,000 Institutions

Trends in investigator-initiated (RO-1 equivalents) vs. NIH targeted research funding



Trends in investigator-initiated (Research Project Grants) vs. NIH targeted research funding

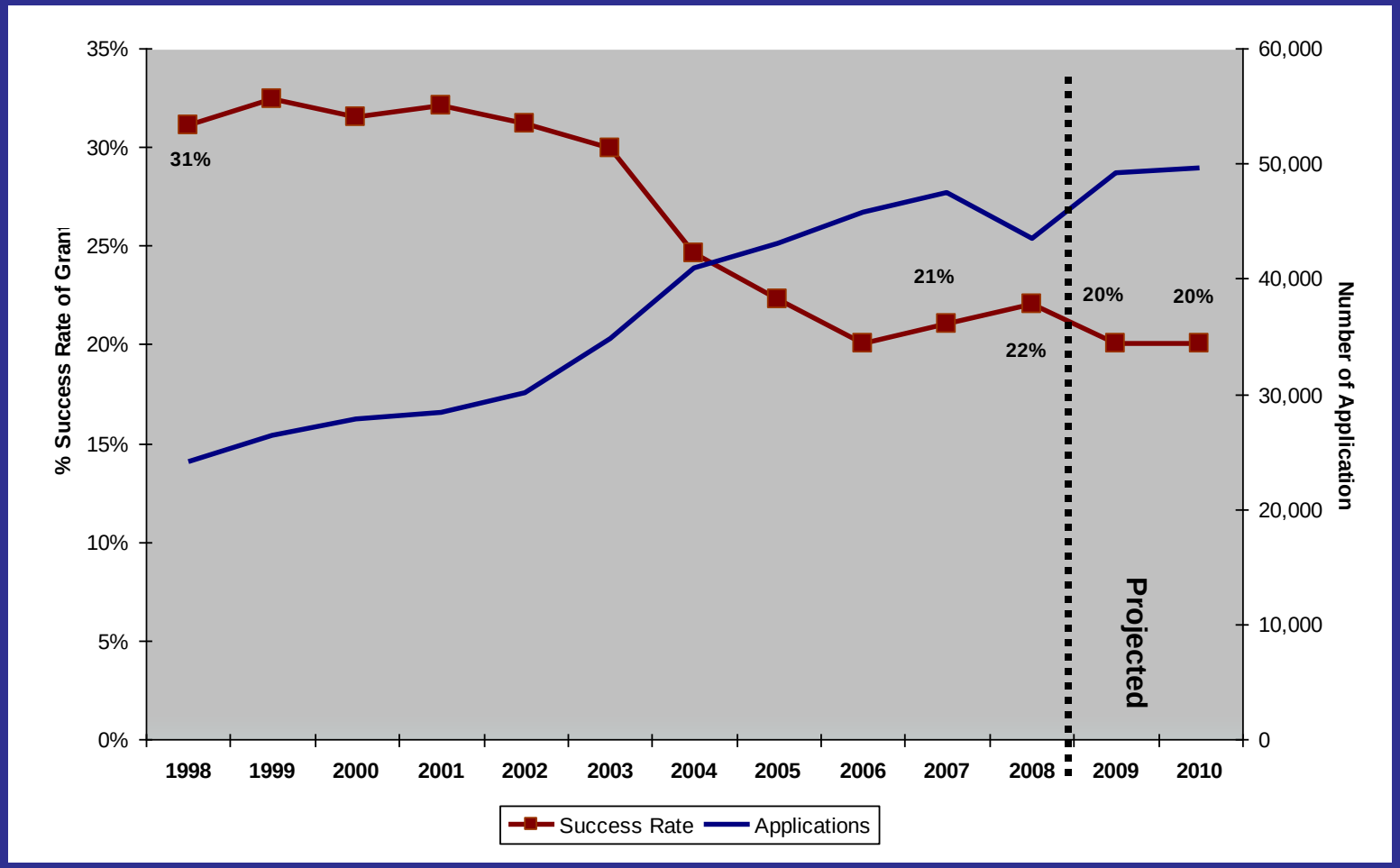


NIH Funding in 2008-2010: Sample of Disease/Research Area

Research/Disease Areas (Dollars in millions and rounded)	FY 2008 Actual	FY 2009 Estimated	FY 2010 Estimated
Acute Respiratory Distress Syndrome	$82	$84	$85
Agent Orange & Dioxin	$13	$14	$14
Aging	$1,965	$2,019	$2,045
Alcoholism	$452	$466	$473
Allergic Rhinitis (Hay Fever)	$6	$6	$6
ALS	$43	$44	$45
Alzheimer's Disease	$412	$423	$428
American Indians / Alaska Natives	$142	$147	$149
Anorexia	$7	$7	$7
Anthrax	$134	$137	$139
Antimicrobial Resistance	$228	$234	$237
Aphasia	$22	$22	$23
Arctic	$22	$23	$23
Arthritis	$232	$238	$241
Assistive Technology	$215	$221	$224

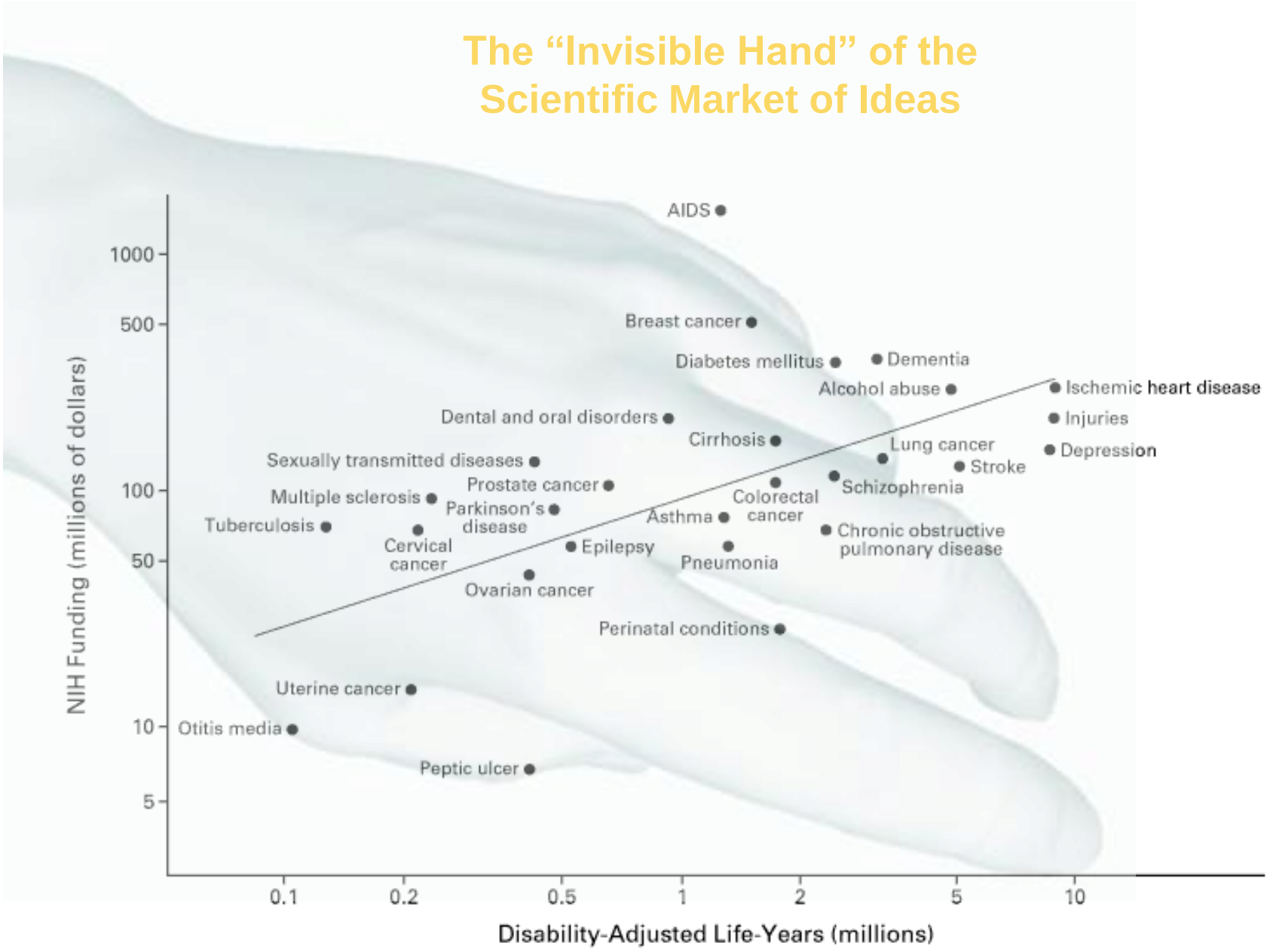
Full list can be accessed at: http://report.nih.gov/rcdc/categories/

National Research Capacity and Demand for Grants Surges at End of Doubling Period, Success Rates Fall then Flatten



* Not adjusted for ARRA

The "Invisible Hand" of the Scientific Market of Ideas



Source: Gross, Cary P., M.D., et al., (1999). The relation between funding by the National Institutes of Health and the burden of disease. New England Journal of Medicine, 340(24); 1881-1887.

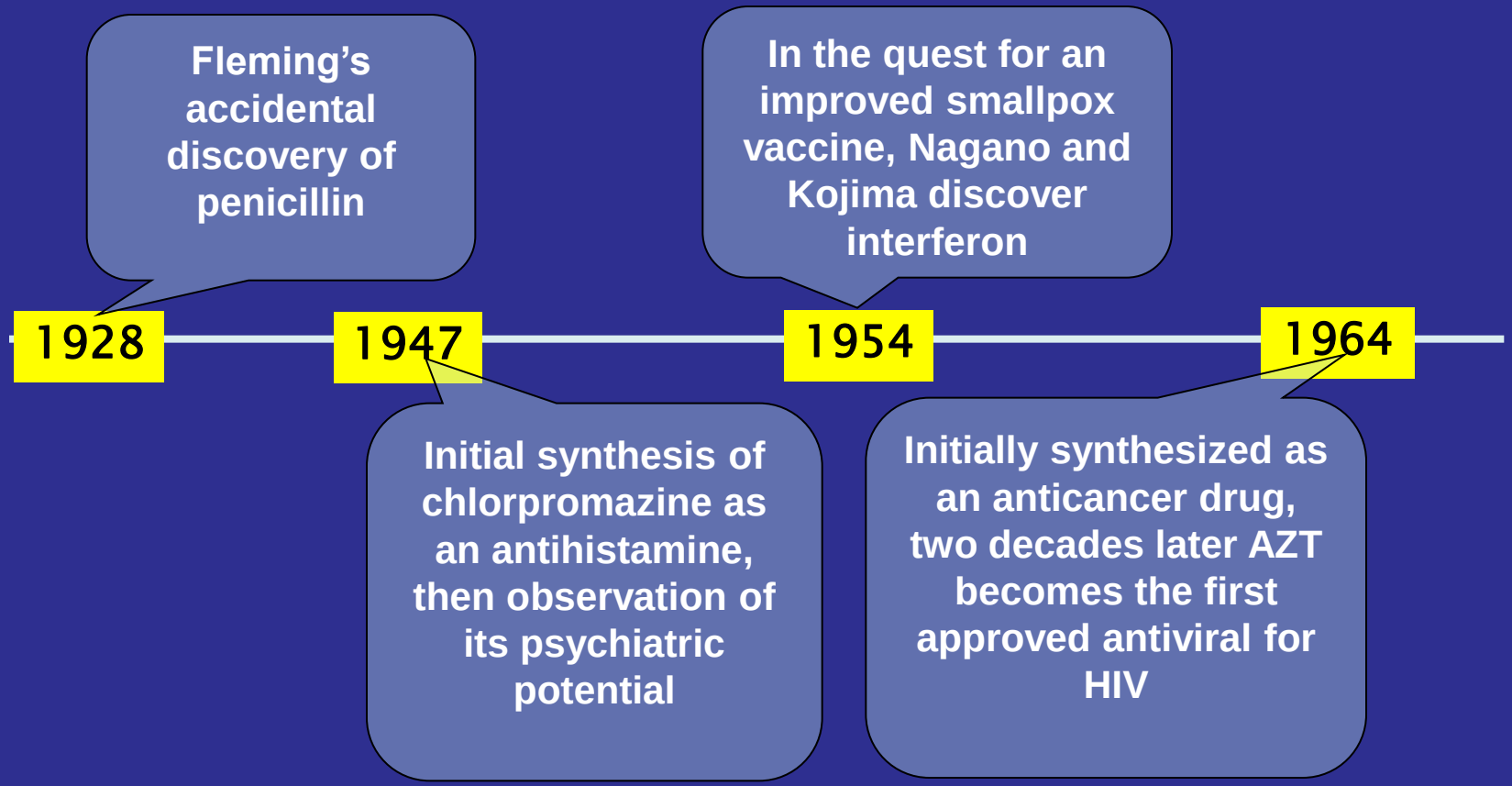
Need to Be Able to Prepare for and Respond to Other Important Public Health Issues

Principles and Imperatives for Priority Setting

- Necessary to ensure that the NIH research portfolio:
  - addresses national public health needs,
  - capitalizes on important scientific opportunities,
  - addresses current and potential needs,
  - attends to research needs for rare and neglected diseases,
  - leverages common interests across IC, and
  - uses resources as effectively as possible.
- Processes for planning and priority setting must:
  - Continue to occur at multiple levels within the timeframe needed for progress in every phase and ultimately for implementation.
  - Be transparent and, along with their outcomes, effectively communicated to all necessary stakeholders.

Principles and Imperatives for Priority Setting: Allowing for Serendipity

Planning and priority setting processes must also acknowledge and be responsive to *the phenomenon of serendipity in scientific discovery*



Improving the Process of Priority Setting: Future Directions

- Key inputs will be better data on the research supported/conducted by NIH and on public health needs.
- Better means for comparing disease and disability burdens (current and anticipated) with information on ongoing scientific efforts and opportunities.
- Comparative analyses utilizing quantitative measures of the quality of science supported across ICs, e.g., range of priority scores, variation in levels of citation of published research.
- Better understanding of what other funders are doing (e.g., foundations, industry)

Improving the Process of Priority Setting: Future Directions

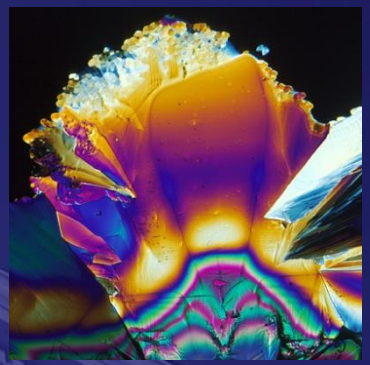
- Trends in and comparisons across ICs in the balance of solicited vs. unsolicited research, use of program projects, center grants, cooperative agreements, contracts.

- Systematic analysis and review of the long-term outcomes of research funded by NIH.

Measures of NIH Success

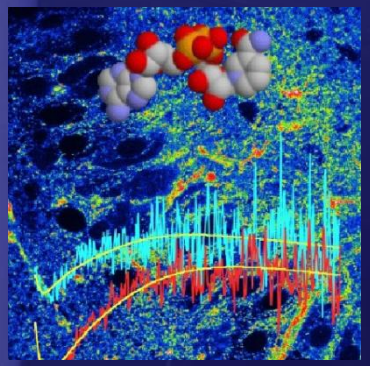
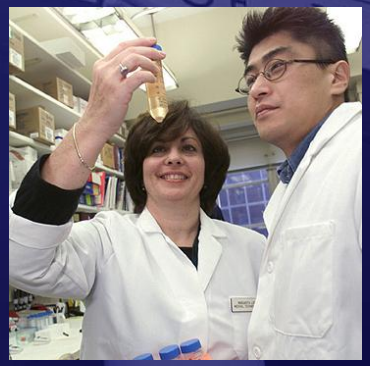
- Discoveries that improve the practice of medicine
- World leadership in science and medicine
- Improved health and life expectancy
- Strength of U.S. universities, medical centers, and industry
- Continued support of Congress and the public






NIH

Transforming medicine and health through discovery


NIH Scientific Management Review Board

Substance Use, Abuse, and Addiction Research Working Group

William Roper, MD, MPH
Dean of the School of Medicine and Vice Chancellor of Medical Affairs, University of North Carolina; CEO of the UNC Health Care System

SUAA

Overview

- Membership
- Context for Deliberations
- Working Group Charge
- Briefings (Past and Future)
- Summary of Public Views
- Questions for Further Investigation
- Additional Considerations
- Future Activities

SUAA

Membership

Non-Federal

William Roper, MD, MPH
(Chair)

Deborah Powell, MD

Eugene Washington, MD

Huda Zoghbi, MD

Norman Augustine
(ad hoc)

Federal

Josephine Briggs, MD

Richard Hodes, MD

Griffin Rodgers, MD

Lawrence Tabak, DDS, PhD

Francis Collins, MD, PhD
(ex officio)

SUAA

Context for Deliberations

Why consider this particular organizational change at this particular time?

Context for Deliberations *(cont…)*

- **Neuroscience research has revealed that addictive substances, including drugs and alcohol:**
  - Differentially affect brain receptors and can result in unique neuropathologies
  - Similarly activate certain physiological pathways including the brain's reward circuit, which can result in compulsive substance use
- **Considering both biological differences and similarities, does the current organization separating research institutes on drug and alcohol use, abuse, and addiction provide optimal infrastructure for supporting these areas of scientific research?**

Context for Deliberations *(cont…)*

- **Social-Political:**
  - **The NIH Reform Act established the SMRB to advise NIH on the use of organizational authorities**
  - **In 2003, the National Academies recommended considering merging NIAAA and NIDA (option of a combined institute of addiction also identified by the Lewin Group in 1988)**
  - **The Drug Abuse Education, Prevention, and Treatment Act of 2001 (S.304) required the DHHS Secretary to request an IOM study to determine whether combining NIDA and NIAAA would strengthen scientific research efforts and increase economic efficiency *(study has yet to be conducted)***

Charge

The Substance Use, Abuse, and Addiction (SUAA) workgroup of the SMRB is convened to recommend whether organizational change within NIH could further optimize research into substance use, abuse, and addiction and maximize human health and/or patient well being.

SUAA

Charge *(cont…)*

The Working Group will consider:

- **Scientific opportunities, public health needs, and new research technologies**
- **Research in these areas under existing NIH structure**
- **Criteria for contemplating changes in the organization and management of NIH today**
- **Strategies for implementing changes in the organization and management of NIH today**
- **Metrics and methodologies that could be used for evaluating the impact of changes in the organization and management of NIH today.**

Briefings: Past

- **SUAA Research at the NIH** - perspectives from NIAAA and NIDA Directors
- **Public Health Needs in SUAA Research** - perspectives from prevention specialists, treatment providers, patient advocates, and policy specialists
- **Science of SUAA** - perspectives from scientists on the mechanisms, pathology, and treatment
- **Alternative Models for Organizing SUAA Research** - perspectives from academia, industry, and the judicial system

Briefings: Future

- **Alternative Models, Past Decisions, and Future Considerations for the Organization of SUAA Research at NIH** - lessons learned from Substance Abuse and Mental Health Services Administration; Alcohol, Drug Abuse, and Mental Health Administration; and the Office of National Drug Control Policy

- **Perspectives from Former Institute Directors**

Advocates FOR Reorganization

- **Science would benefit from synergy of commonalities:**
  - **Emerging scientific research indicate similar reward pathways underlie compulsive behavior**
  - **Alcohol and drug abuse often begins in adolescence with similar early risk factors**
- **High prevalence of drug users also use alcohol, suggesting both scientific and policy justification**
- **Segregation of disciplines create public health gaps**

Advocates FOR Reorganization *(cont…)*

- **Reorganization, particularly merging, would:**
  - **Create synergy for advancing the science of substance use, abuse and addiction**
  - **Increase flexibility in cross-training**

Advocates AGAINST Reorganization

- **Reorganization would create research gaps in understanding:**
  - **Multiple organ targets of alcohol**
  - **Unique factors underlying abuse and addiction**
- **Contextual and socio-cultural differences warrant separate, focused research efforts**
- **Lack of compelling evidence to suggest reorganization would improve treatment, prevention, research, and/or training**
- **Current organization mirrors the separation of professional and scientific associations**

Advocates AGAINST Reorganization *(cont…)*

- **Reorganization, particularly merging, would:**
  - **Decrease emphasis on effects of alcohol on multiple organ targets**
  - **Jeopardize priority/budget of alcohol-related research**
  - **Create organizational/administrative obstacles and reversals**

Questions for Further Investigation

- **How is the science being (or not being) served by the current organization?**
  - **Are any areas of science neglected?**
  - **Are gaps in public health created (e.g., poly-substance abusers)?**
  - **Are there sufficiently common biological pathways warranting a more integrated scientific approach?**

SUAA

Questions for Further Investigation *(cont…)*

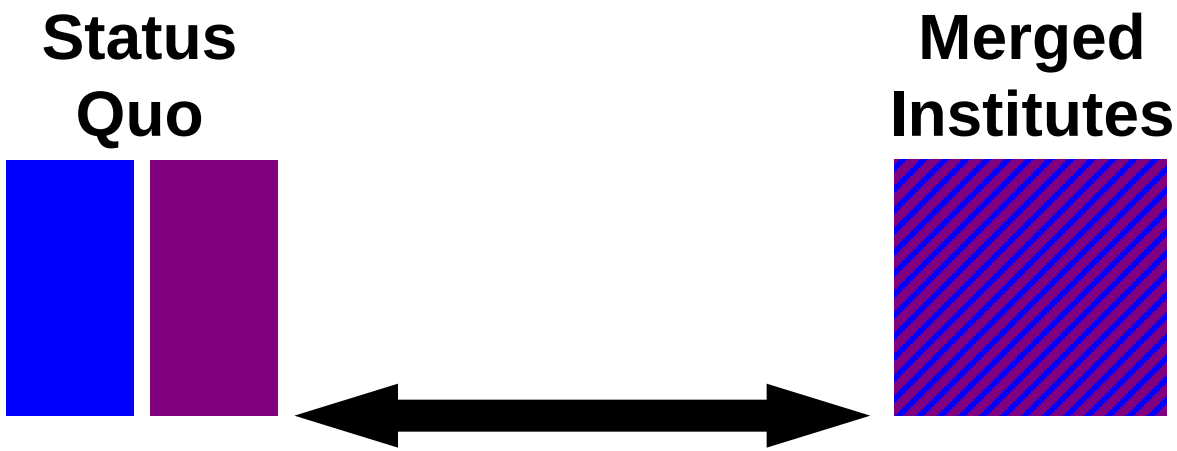
- **What research is (or is not) already being conducted by NIH in the field of addiction?**
  - **What is the scientific and funding portfolio of addiction-related research across ICs?**
    - **% of total budget?**
  - **Are there existing collaborations across ICs?**
    - **Examples from the intramural program?**
    - **Examples from the extramural program?**

Questions for Further Investigation *(cont…)*

- **How do other countries/organizations optimize the organization of SUAA research?**
  - **What are the alcohol-related research priorities in other countries?**
  - **How do international research agencies organize around alcohol and drug abuse?**

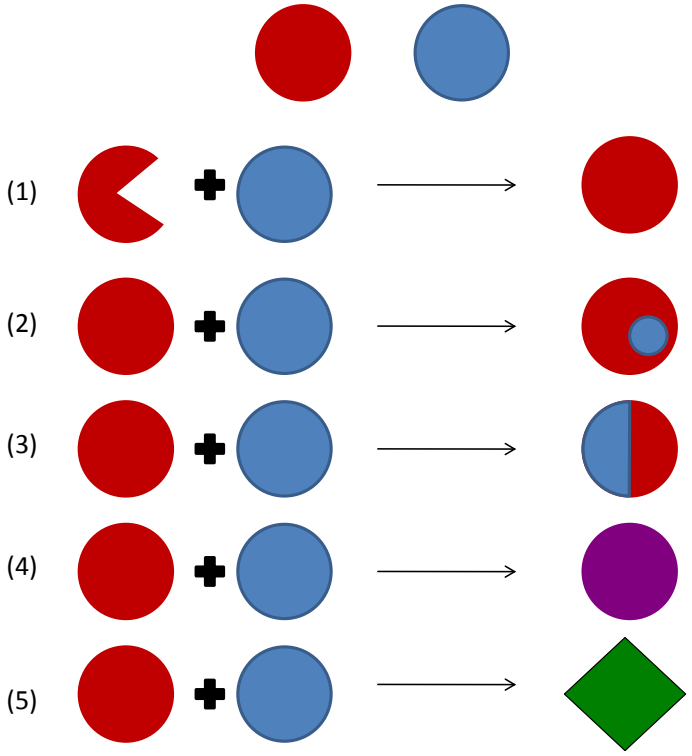
Additional Considerations

- **Initial dialogue focused on either the “status quo” or “merging” Institutes**



Additional Considerations *(cont…)*

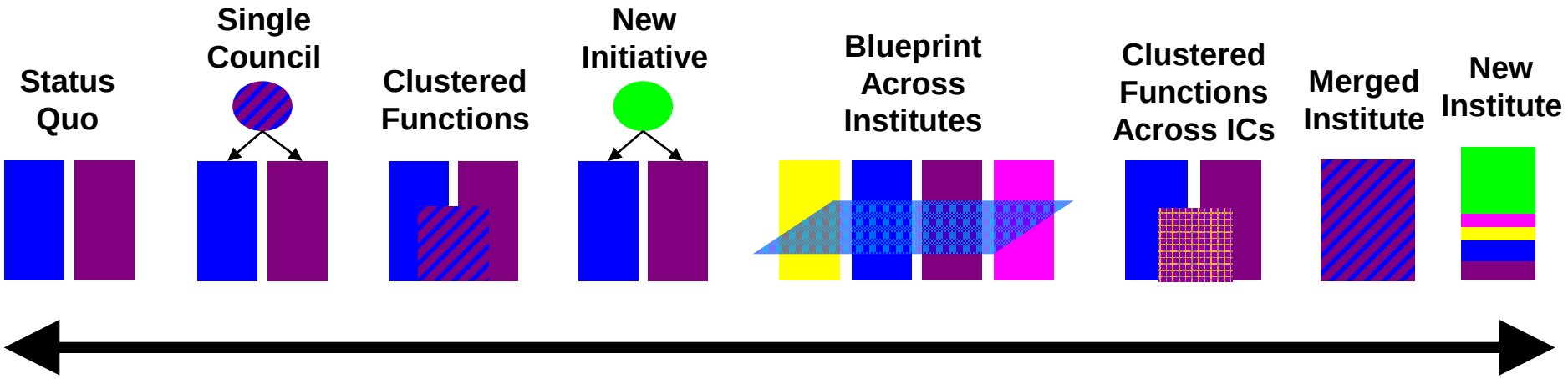
- **However, it is important to consider that mergers can take multiple forms and have multiple options**



SUAA

Full Spectrum of Options

- **Each option needs to be carefully considered to maximize functional integration**



Future Activities and Next Steps

- Additional briefings
- Collect data to answer questions for further investigation
- Evaluate full spectrum of organization models
- Brief relevant IC Councils

DISCUSSION

NIH Scientific Management Review Board

NIH Intramural Research Program Working Group

Arthur Rubenstein, M.B.B.Ch.
Executive Vice President of the University of Pennsylvania for Health System and Dean of the University of Pennsylvania School of Medicine

Overview

- Membership
- Historical Context
- Working Group Charge
- Briefings (Past and Future)
- Summary of Findings to Date
- Future Activities

Membership

Non-Federal

Arthur Rubenstein, MBBCh *(Chair)*

Gail Cassell, PhD

Solomon Snyder, PhD

Norman Augustine *(ad hoc)*

Federal

Anthony Fauci, MD

Stephen Katz, MD, PhD

Elizabeth Nabel, MD

Francis Collins, MD, PhD *(ex officio)*

NIH Intramural Research Program: A Strong Foundation

- **Since its inception, the NIH has supported an intramural research program (IRP) with a unique set of characteristics**
  - **Size**
  - **Retrospective model of scientific review**
  - **A relatively stable budget (until recently)**
- **To date, the IRP at NIH has demonstrated numerous accomplishments, including:**
  - **Transformative advances in biomedical research**
  - **Training the leaders of our nation's academic health centers**
  - **Serving as a trusted source of medical information**
  - **Facilitating numerous collaborative interactions**

Charge

The NIH Intramural Research Working Group of the SMRB is convened to recommend whether any change in the organization and/or management of NIH intramural research could further optimize the opportunities available in a central research program at NIH and maximize human health and/or patient well being.

Charge *(cont...)*

Given that recent internal assessments have pointed towards the urgency of addressing the fiscal vitality of the NIH Clinical Center, the Working Group will carry out the following tasks in order:

- **Analysis of and recommendations regarding the fiscal sustainability and utilization of the NIH Clinical Center**
- **Analysis of and recommendations regarding the optimal organization of the overall NIH intramural research program**

Re-examining the NIH Clinical Center

- **Historically, the NIH Clinical Center (CC) has provided a versatile clinical research environment enabling the NIH mission to improve human health**

Briefings to Date

- **Concerns and Current Status of the NIH Clinical Center** - overview on current fiscal challenges and options for future sustainability
- **The NIH Clinical Center: Mission, Function, Capabilities, and Vision for the Future** - perspectives from distinguished NIH investigators and advisers
- **Business Models for Hospital Management** - perspectives from research hospital administrators
- **Collaborations between Extramural and Intramural Communities**
  - **limitations and possibilities**
  - **existing collaborations**

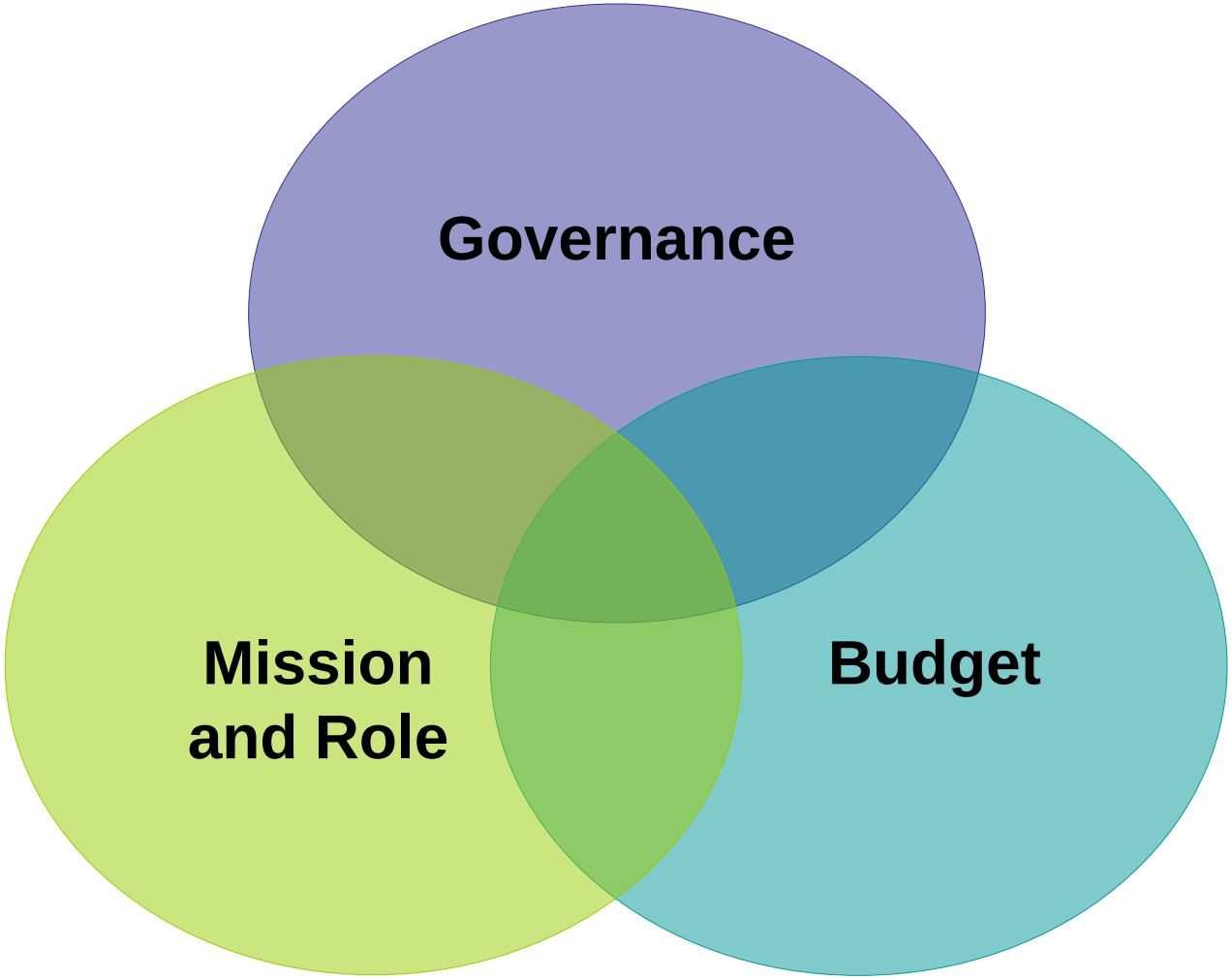
Summary to Date: Benefits

- **Research Flexibility**
  - **Investigators free to devote full attention on research**
  - **Permits nimble responses to emergent scientific opportunities and public health needs**
  - **Patient care is fully funded**
  - **Staff has immediate access to cutting-edge technologies**
  - **Provides opportunities to conduct high-risk trials for life threatening diseases**
  - **Permits failure**

Summary to Date: Benefits *(cont...)*

- **Houses a critical mass of highly skilled individuals**
- **Plays a critical role in first-in-human studies and rare disease research**
- **Supports longitudinal studies**
- **Serves as a laboratory to study human biology and pathology**
- **Fosters distinctive training opportunities**
- **Provides a visible window to NIH for the public and policy makers**

Summary to Date: Challenges

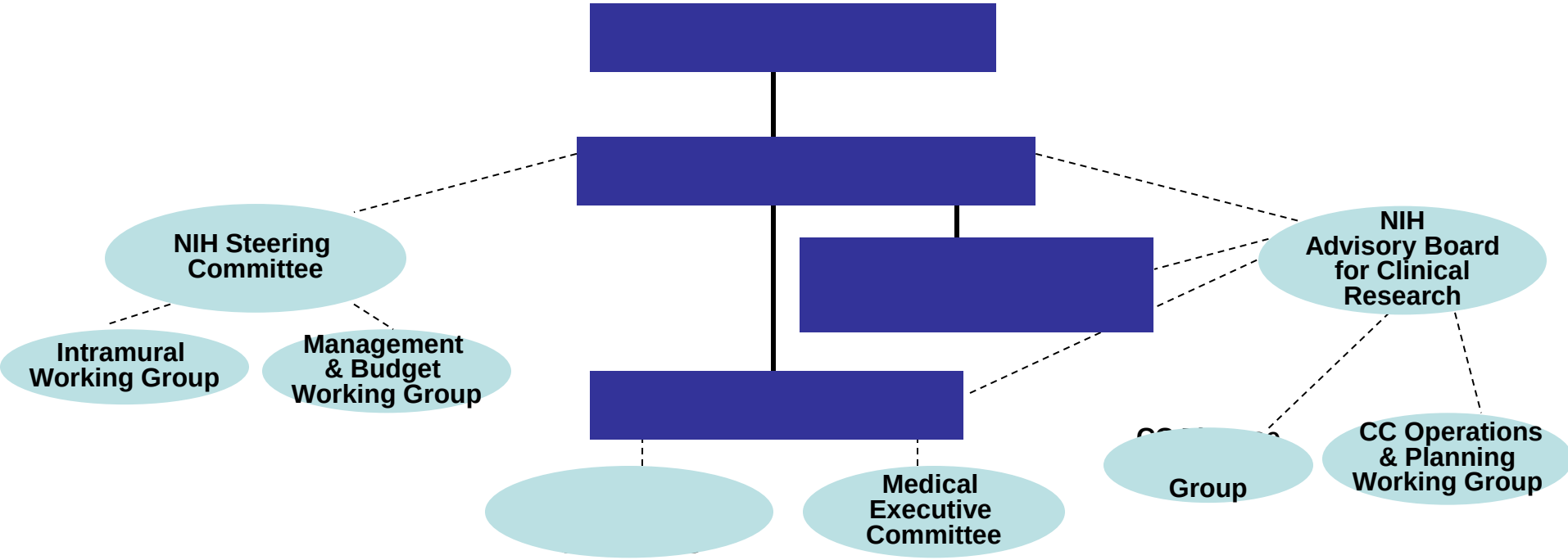


Challenges: MISSION

- Perceived lack of prioritization of and commitment to funding clinical research at the CC
- Barriers to partnerships and leveraging resources (e.g., barriers to intra-/extramural collaborations, intellectual property)
- Barriers to recruitment, mentorship, and retention of investigators

Challenges: GOVERNANCE

- **Lack of trans-NIH vision for priority setting in clinical research**
- **Complexity in administrative approval processes**



Challenges: BUDGET

- Increasing costs of CC associated with healthcare inflation - current “School tax” method does not keep up with inflation
- Instability of CC funding
- Cost shifts have had unintended and undesirable consequences (e.g., significantly reduce use of CC use by ICs)
- Budget mechanism does not support outside investigators’ use of CC

Emerging Findings

- ***Clinical Center as a National Resource*. CC should be a state-of-the-art national resource; resources should be optimally manage to enable both internal and external investigators to use the CC**
- ***Streamlined Governance Structure.* A clear, coherent plan for clinical research at the Clinical Center and a simplified governance structure are need to oversee appropriate use and adequate funding**
- ***Stable, Responsive Budget Underpinned by Priority Setting Process.* Budget should be linked to a strong planning process and be stable, equitable (in source and distribution), effective in attracting and supporting a high quality workforce, and should assure efficient use of the CC**

Current Activities: Examining Potential Funding Models

- **Identifying attributes of potential models:**
  - **Source of funds and locus of control**
  - **Legal and regulatory considerations**
- **Evaluating potential for models to provide:**
  - **Stability and continuity of funding**
  - **Responsiveness to trends in science and health**
  - **Incentives for IC collaboration**
  - **Ability to position the CC as a national resource, both internally and externally**
  - **Resiliency in the face of economic constraints**

Future Activities

- Additional briefings
- Evaluate potential strategies for enhancing the role of the Clinical Center as a national resource, including various models for governance and funding
- Brief the Advisory Board for Clinical Research
- Host public forum with relevant stakeholders

DISCUSSION