



National Institutes of Health
Turning Discovery Into Health

ATTACHMENT 6

Scientific Management Review Board

SMRB Working Group on Approaches to Assess the Value of Biomedical Research Supported by NIH

Gail Cassell, PhD

Chair, AAVOBR Working Group

SMRB

AAVOBR WORKING GROUP – September 18, 2013

Today's Agenda

- **Initial findings and conclusions**
 - I. **Introduction**
 - II. **Overview**
 - III. **Findings and Conclusions**
- **Public comment**
- **SMRB member discussion**
- **Next steps**

Charge Issued by the NIH Director

NIH requests that the SMRB identify appropriate parameters and approaches for assessing and communicating the value of biomedical research supported by NIH.

—Presentation to SMRB on July 11, 2012

Working Group Roster

NON-FEDERAL

- Gail Cassell, PhD (*Chair*)
- Norman Augustine
- Hon. Daniel Goldin
- Gilbert Omenn, MD, PhD
- Arthur Rubenstein, MBBCh

FEDERAL

- Alan Guttmacher, MD
- Richard Hodes, MD
- Stephen Katz, MD, PhD
- Griffin Rodgers, MD, MACP
- Martha Somerman, DDS, PhD

Summary of Key Points Identified by Working Group

- Working Group members believe that the value of NIH is incontrovertible. Its work has led to improvements in the health and well-being of all Americans and many others around the globe.
- Though some of what NIH produces is easy to measure, these markers of progress do not begin to fully capture the wealth of NIH's contributions to the world.
- Now is an ideal time for NIH to capitalize on the ongoing revolution in data collection and analysis by intensifying its efforts to systematically, comprehensively, and strategically assess its value. Results can then be used to demonstrate accountability, increase public awareness, and optimize impact.

Summary of Key Points Identified by Working Group *(cont.)*

- **There are many compelling reasons for NIH to continually improve its ability to assess value** (e.g., emergence of better data and tools, accountability to the public).
- **However, assessing NIH's value is complicated due to a number of factors** (e.g., difficulty demonstrating attribution).
- **NIH should strengthen its assessment of value by undertaking a coordinated, comprehensive strategy to:**
 - Identify representative study topics;
 - Improve its data infrastructure; and,
 - Determine appropriate methodologies based on purpose, audience, and study topic.

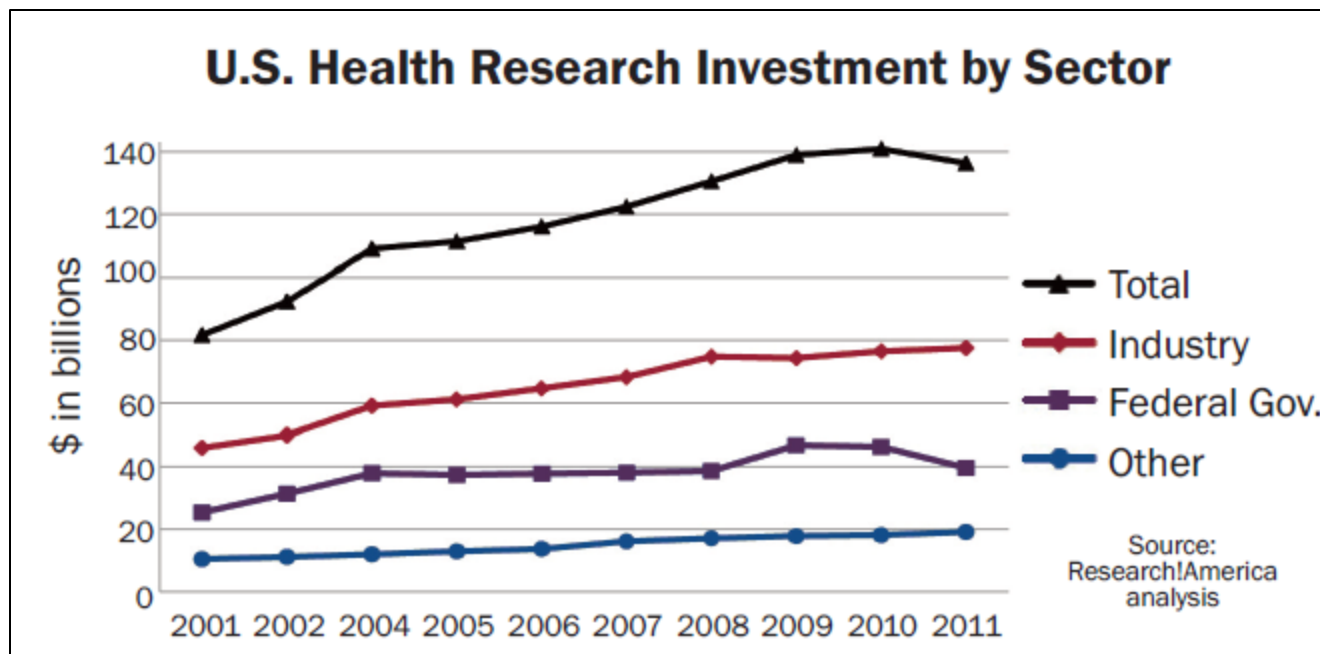
I. INTRODUCTION

- NIH is responsible for investing public funds in biomedical research and should use funds effectively
- There are increasing opportunities and expectations to improve assessment efforts
- Assessment results can improve priority-setting and decision-making processes at NIH, but it is first necessary to ensure assessments are sound

II. OVERVIEW

A. The biomedical research enterprise

- U.S. leads the world in biomedical R&D (\$130B), involving multiple stages over many years, fields, and funding from many sources



For more information, see http://www.researchamerica.org/research_investment.

II. OVERVIEW

B. NIH's role in the biomedical research enterprise

- In FY 2012, NIH had a budget of \$30.86B and used it to:
 - **Support** research through funding, training, and infrastructure development
 - Supported products are near-term (grants) and long-term (infrastructure and workforce development)
 - **Conduct** research

II. OVERVIEW

C. The NIH mission

- The NIH mission is to “seek fundamental knowledge about the nature and behavior of living systems and the application of that knowledge to enhance health, lengthen life, and reduce illness and disability”
- Because it is not in NIH’s purview to provide or regulate health services, NIH must partner with many other players (especially sister agencies within HHS)

II. OVERVIEW

D. Capturing the value of NIH's achievements

Three value “streams” based on NIH mission:

- **The value of fundamental knowledge**
 - Increased understanding of biological and behavioral systems
- **The value of public health effects**
 - Application of research findings into clinical and public health practice
- **The value of broader societal impacts**
 - International competitiveness, technological advancements, increased scientific literacy, economic benefits, international collaboration, and others

II. OVERVIEW

E. Challenges to accurately assessing value

- **Differing definitions of value**
- **Proper attribution is very difficult**
 - Time considerations
 - Discovery to health impact often takes many years
 - A discovery that seems to have little significance today may prove transformational in the future, and a single finding may have implications for numerous subsequent lines of inquiry
 - Complexity of the biomedical research and public health enterprises
 - Multiple actors in the scientific and public health ecosystems have different roles to play in realizing the benefits of biomedical research

II. OVERVIEW

E. Challenges: The Case of Restriction Enzymes

Fundamental Knowledge

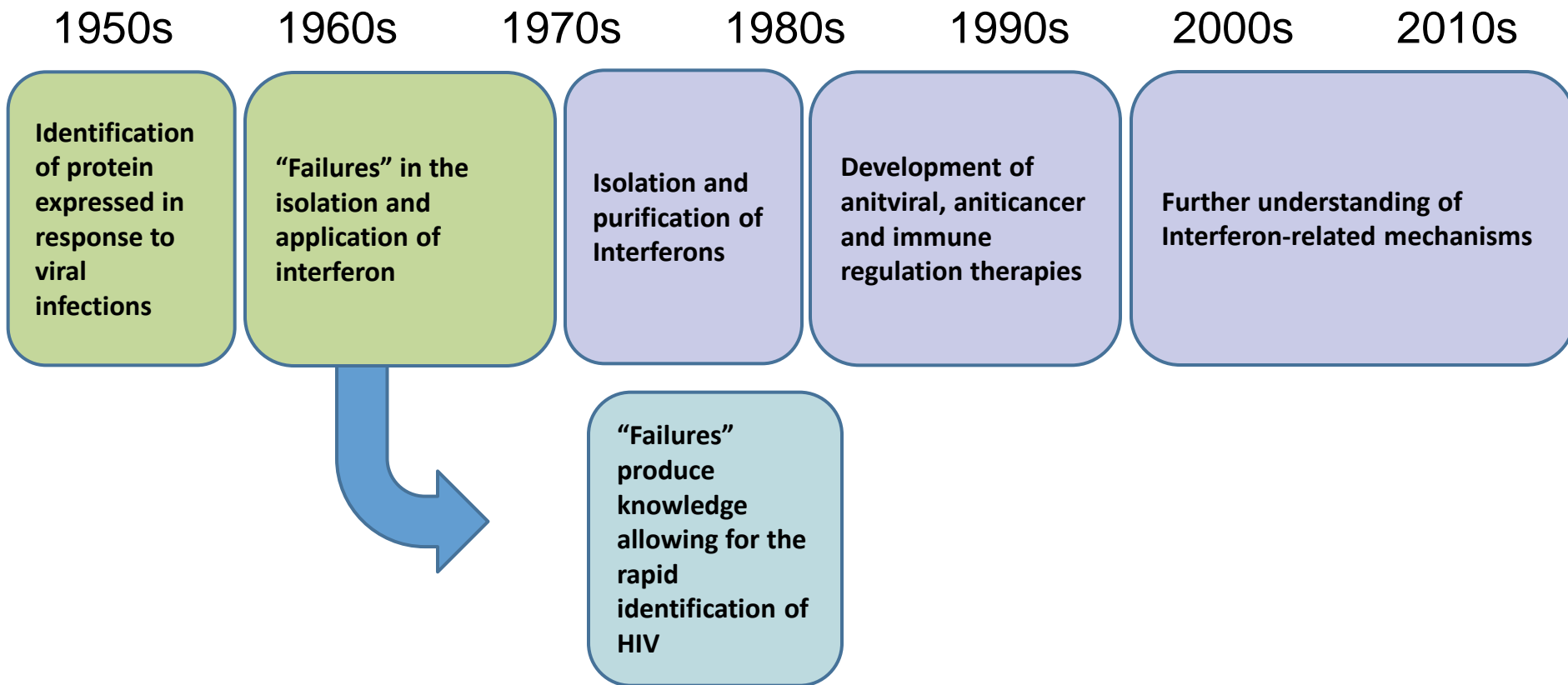
Health Applications

Broader Societal Impacts

	1960s	1970s	1980s to Today
Fundamental Knowledge	<p>Discovery of DNA “cut-and-paste” enzymes*</p> <p>*multiple U.S. and non-U.S. research groups funded by NIH, NSF, Fonds National Suisse de la Recherche Scientifique, among others</p>	<p>Initial development of cloning techniques</p> <p>1978 Nobel Prize (Arber, Nathans*, & Smith*)</p> <p>*NIH-supported</p>	<p>Transgenics: Introducing human mutations into animals to understand disease</p> <p>Gene Function: e.g., Understanding roles of proteins in cellular pathways</p>
Health Applications			<p>Disease treatments, including recombinant human insulin for diabetes and gene therapy</p> <p>Disease diagnostics, including phenylketonuria, cystic fibrosis, leukemia, and AIDS</p>
Broader Societal Impacts			<p>Biotech industry: Foundational technology has enabled billions in industry profits</p> <p>Criminal forensics: First generation DNA-fingerprinting</p>

II. OVERVIEW

E. Challenges: The Case of Interferons



II. OVERVIEW

F. The purpose of assessing NIH's value

- **Accountability to the public**
 - NIH can demonstrate whether money and other resources have been used efficiently and effectively
- **Raising public awareness of NIH's value**
 - NIH can make the case for research investments and enhance the public's understanding of the scientific process
- **Management of NIH's portfolio and activities**
 - By better understanding the results of its activities, NIH leadership can make ever-more informed decisions with an eye toward enhancing its value to the American public

III. Findings and Conclusions

Considerations – Key Finding #1

- **Finding:**

- **NIH's value is derived from producing knowledge that can be applied to improve public health.**

- **Context:**

- The preponderance of NIH's public health and broader societal value stems first from the generation of knowledge. Therefore, a proper understanding of the value of biomedical research will include the role and value of knowledge generation.
- Knowledge has innumerable benefits. Whether by influencing medical practice, or enhancing international relations, knowledge generation has profound implications for individuals and for society.
- However, placing empirical value, especially a dollar figure, on knowledge is difficult.

III. Findings and Conclusions

Considerations – Key Finding #1 *(cont.)*

- **Finding:**

- NIH's value is derived from producing knowledge that can be applied to improve public health.

- **Potential conclusion(s):**

- Assessments of NIH's value should attempt to draw clear connections between the generation of knowledge and its application to health and broader societal impacts. For example, NIH should strive to educate its stakeholders about the many ways basic discovery is critical to biomedical research and its ultimate value to health.

III. Findings and Conclusions

Considerations – Key Finding #2

- **Finding:**

- **Many factors need to be considered in order to accurately determine NIH's contribution to a particular outcome.**

- **Context:**

- NIH's role is to generate knowledge, not to provide health services, regulate public health, or change behaviors. Because these responsibilities fall to many other actors in the public health ecosystem, they will have great influence on NIH's value.
- Many of NIH's efforts—including funding research, providing for training, or developing infrastructure—are aided by partners; therefore, NIH is not always the sole source of support.
- There is often a significant lag time between a discovery and its application.

III. Findings and Conclusions

Considerations – Key Finding #2 *(cont.)*

- **Finding:**

- **Many factors need to be considered in order to accurately determine NIH's contribution to a particular outcome.**

- **Potential conclusion(s):**

- Credible, interpretable, and useful assessments of the value of NIH should:
 - Acknowledge and determine to the extent possible the role of other players in the advancement and adoption of research findings or other outcomes of interest.
 - Attribute outcomes entirely to NIH only when this is proven to be the case.
 - Establish a timeframe that is broad enough to include sufficient time for discovery to be applied.

III. Findings and Conclusions

Considerations – Key Finding #3

- **Finding:**

- **NIH affects and is affected by many participants in the scientific and public health ecosystems; therefore, there are many stakeholders to consider in any attempt to assess its value.**

- **Context:**

- There are many different audiences for assessments of value, including the public and their representatives, HHS, public health workers, the scientific community, and NIH staff.
- Assessment efforts may have important ramifications for some NIH stakeholders, whether it be the conduct of the study itself (e.g., reporting requirements for researchers) or the findings (e.g., the identification of new research directions).

III. Findings and Conclusions

Considerations – Key Finding #3 *(cont.)*

- **Finding:**

- **NIH affects and is affected by many participants in the scientific and public health ecosystems; therefore, there are many stakeholders to consider in any attempt to assess its value.**

- **Potential conclusion(s):**

- NIH's assessment and communication strategies should reflect many voices, including NIH leadership and program staff, researchers, research institutions, patients, practitioners, other Federal agencies, and the private sector. To this end, NIH should regularly seek input from stakeholders to inform its assessment efforts.
- NIH should give careful consideration to the consequences of its assessment efforts.
- NIH should seek ways to partner in its attempt to assess its value (e.g., data linkages) when appropriate.

III. Findings and Conclusions

Assessment Approaches #1 – Study Topics

- **Finding:**

- Numerous attempts to assess aspects of NIH's value have been undertaken by NIH and by many of its stakeholders, but these efforts have not been comprehensive, systematic, or coordinated.

- **Context:**

- Because these studies have not been well coordinated, they do not necessarily reflect the full spectrum of NIH activities.
- Value and impact assessments can be criticized for focusing on the most successful projects rather than reflecting a representative snapshot of NIH-funded research.

III. Findings and Conclusions

Assessment Approaches #1 *(cont.)*

- **Finding:**

- Numerous attempts to assess aspects of NIH's value have been undertaken by NIH and by many of its stakeholders, but these efforts have not been comprehensive, systematic, or coordinated.

- **Potential conclusion(s):**

- Based on pre-established principles, NIH should support systematic assessment studies that are strategically selected to encompass the full spectrum of NIH activities and processes.
- A trans-NIH Committee on Assessments should be established to coordinate agency efforts in assessing its value, including the design of pilot studies to test the framework for study selection.

III. Findings and Conclusions

Assessment Approaches #2 – Data Needs

- **Finding:**

- **There is insufficient data collection, storage, and linkage between data sets to conduct thorough assessments of value.**

- **Context:**

- Valid assessments of value rely on sound data, verifiable relationships between data sets, and rigorous analysis.
- While NIH's activities result in diverse products and impacts, views vary on which indicators best reflect these products and impacts.
- NIH's data infrastructure was built primarily to manage grants and contracts during their life-cycle, not to track outcomes.
- Ongoing efforts to improve NIH's data infrastructure for the purpose of tracking outcomes can be further enhanced by increased coordination across the agency.

III. Findings and Conclusions

Assessment Approaches #2 *(cont.)*

- **Finding:**
 - **There is insufficient data collection, storage, and linkage between data sets to conduct thorough assessments of value.**
- **Potential conclusion(s):**
 - NIH should capitalize on its many efforts in “big data” to include improving its own data infrastructure for monitoring and assessing itself. This would include efforts in:
 - Identifying and gaining consensus on a core set of indicators to be included in its data infrastructure.
 - Linking its own data infrastructure with that of its many partners in the science and public health ecosystems (e.g. CDC, USPTO, FDA).
 - Improving the quality and integrity of core data and linkages through ownership and governance.
 - To accomplish this in a strategic, coordinated fashion, the trans-NIH Committee on Assessments should interface with recently established NIH Big Data governance activities.

III. Findings and Conclusions

Assessment Approaches #3 – Methodologies

- **Finding:**

- **Sophisticated and rigorous methodologies are needed to fully capture the value of NIH. Of the many assessments that have been undertaken, no single approach has proven entirely satisfactory.**

- **Context:**

- There is no ideal methodological approach to assessing the value of NIH.
- Narratives built from well-designed case studies are particularly effective at illustrating the importance and wide-ranging impacts of biomedical research.
- A wide variety of complementary approaches should be employed in the assessment and communication of value.
- Whether retrospective or prospective, quantitative or qualitative, methodological approaches should be determined by the purpose, context, and intended audience to which the assessment is directed.

III. Findings and Conclusions

Assessment Approaches #3 *(cont.)*

- **Finding:**

- **Sophisticated and rigorous methodologies are needed to fully capture the value of NIH. Of the many assessments that have been undertaken, no single approach has proven more effective than others.**

- **Potential conclusion(s):**

- Analytic approaches should balance numbers with narratives, illustrating the complexities of progress, such as the time-dependence of R&D and the pivotal roles of other actors in the biomedical enterprise.
- NIH should adopt a systematic approach to designing case studies which can tell compelling and accurate stories of NIH's role in turning discovery into health.
- The recommended trans-NIH Committee on Assessments should develop a decision tree to help guide methodological approaches for NIH assessments based on a number of nested factors beginning with the kind and purpose of the evaluation. This decision tree should be updated at yearly intervals to reflect new tools or approaches.

Next Steps: Working Group Activities

- **Working Group activities**

- Receive feedback from SMRB members and the public during today's teleconference
- Refine draft findings and conclusions
- Finalize agenda for October 24–25 SMRB meeting

Next Steps: SMRB

- **October 24–25 SMRB meeting in Bethesda**
 - Receive input from stakeholders, including:
 - Broader societal and high-level experts
 - Public and patient advocacy representatives
 - Non-U.S. assessment experts
- **December 18 SMRB teleconference (10:00AM–12:00PM)**
 - Receive report
 - Vote on report findings and conclusions