

SMRB Working Group on the Value of Biomedical Research

Gail Cassell, PhD
Chair, VOBR Working Group

SMRB

VOBR WORKING GROUP – June 4, 2013

Working Group Roster

NON-FEDERAL

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

FEDERAL

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

– 2 –

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Working Group Activities to Date

- **July 11, 2012 (SMRB):** NIH Director issues charge to SMRB regarding assessing the value of biomedical research
- **Sept–Dec 2012:** Compilation and analysis of relevant literature; discussion of basic evaluation framework
- **January 14, 2013 (SMRB):** SMRB meeting includes panel session focused on the economic value of biomedical research; VOBR Working Group members review relevant literature
- **March 2013:** Briefings by NIH staff on data collection and analysis tools and technology transfer; draft framework for tools and metrics for assessing value
- **April 2013:** Draft outline of report; discuss types of value and major elements of charge
- **May 2013:** Prepare questions for June 4 panel discussions

– 3 –

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Deliberative Process

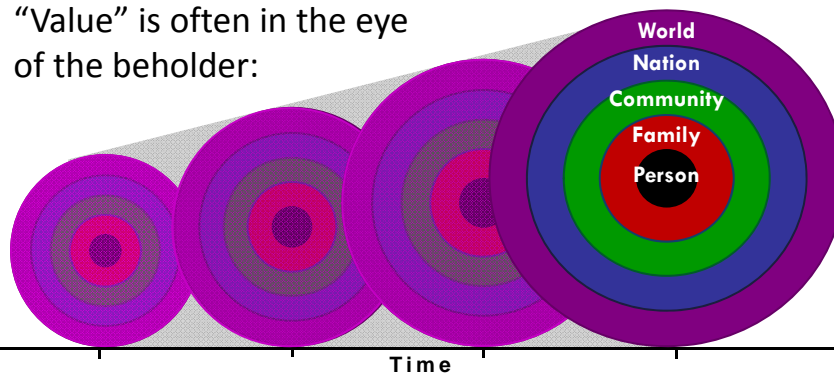


– 4 –

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Definition of Value

“Value” is often in the eye of the beholder:



What constitutes value, who makes that determination, and how can it be observed across time?

– 5 –

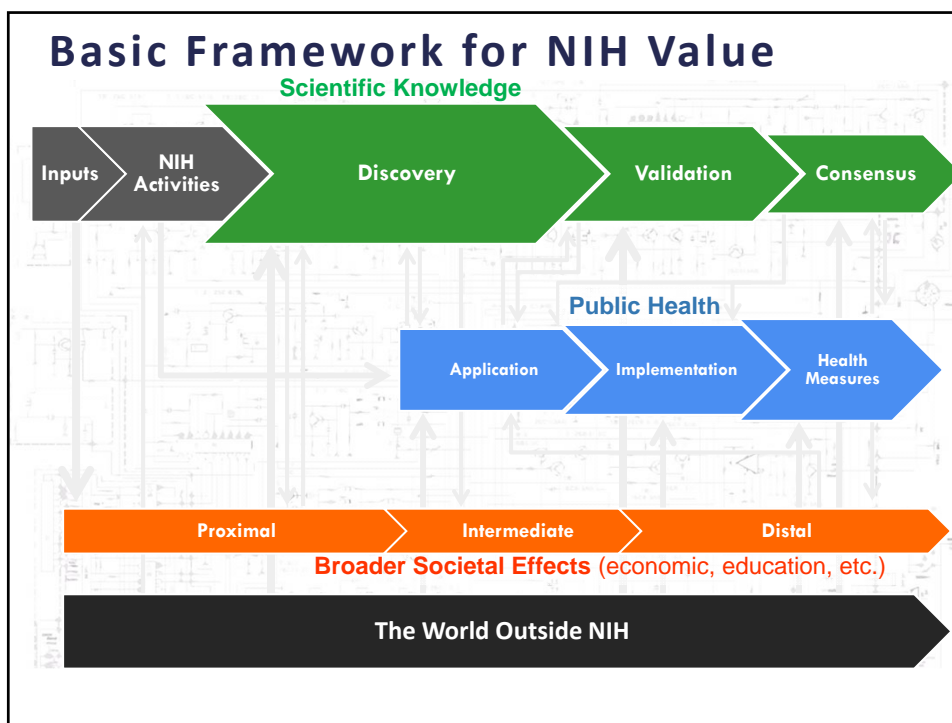
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Three Types of Value

- NIH’s 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 the burdens of illness and disability**.
- The Working Group has divided areas of biomedical research value into three streams:
 - Scientific knowledge
 - Public health
 - Broader societal impact

– 6 –

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Major Elements of the Charge

1. **Principles** that should underlie assessments of value
2. Advice regarding sound **methods and strategies** for assessing value
3. Advice regarding **study questions and selection of study topics** (e.g., case studies)

*Charge Element 1: Principles***Guiding Principles, Limitations, & Caveats**

*Why does NIH need to better assess its value?
What can we accomplish with this effort?*

The SMRB is tasked with advising NIH on the objectives of value assessments and the realistic boundaries of assessing, attributing, and communicating value.

- 9 -

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*Charge Element 1: Principles***Principles Underlying Value Assessment**

- Attribution
- Causality
- Precision
- Comprehensiveness
- Disclosure of assessment limitations
- Reflect values of society
- Other?

- 10 -

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*Charge Element 1: Principles***Challenges with Assessing Value**

Assessing the value of biomedical research is difficult due to challenges with assigning attribution and causality.

- Multiple factors and sectors influence the **downstream effects** of NIH activities.
- It is difficult to estimate and account for the **lag time** between research and impact.
- There are myriad challenges in collecting and analyzing data that **accurately capture the outcomes** of NIH activities.

Is there anything unique for NIH, compared with other R&D agencies, in these challenges?

- 11 -

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*Charge Element 1: Principles***Common Elements of Value Assessments**

- Many models for assessing value have been developed for different contexts
- It would be useful to develop a generic model that can be customized and adapted to various study questions
- Identification of the critical components of such a model should be part of the Working Group's findings



- 12 -

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Charge Element 2: Methods & Strategies

Methods & Strategies

How should we measure value?

The SMRB is tasked with identifying a set of metrics and strategies (established and emerging) that are most appropriate for this task.

Types of Value	OUTPUTS						OUT-COMES
	Short-term		Medium-term		Long-term		
Science	DISCOVERY		VALIDATION		CONSENSUS		Fundamental Scientific Knowledge
	Indicators	Measurement and assessment tools	Indicators	Measurement and assessment tools	Indicators	Measurement and assessment tools	
	Conference abstract presentation	NIH Research Performance Program Report (RPPR), <i>Tool link</i>	Patent	USPTO Analysis of Patent Applications (linking publications to patents), RPPR, FDA Orange Book, etc.	FDA approval of New Drug Application (NDA)	FDA Orange Book	
Public Health	APPLICATION		IMPLEMENTATION		HEALTH MEASURES		Living longer, healthier lives
	Indicators	Measurement and assessment tools	Indicators	Measurement and assessment tools	Indicators	Measurement and assessment tools	
	Advanced testing of evidence-based diagnostic	RuPORTER, LuPORTER, RPPR, Patent applications (USPTO), IP-related data, FDA, <i>Tool link</i>	Disruptive medicine	FDA approvals, Industry Reports	Burden of Disease Profile of Life	DALYs, DALYs, WHODS, etc. - CDC (DHHS), WHO, survey data (IHAP, IHMIS, etc.)	
Broader Societal Impacts	PROXIMAL		INTERMEDIATE		DISTAL		Scientific literacy public ↓ Healthcare-related cost ↑ Productivity ↑ Capacity for Innovation ↑ Global R&D competitiveness Sci. diplomacy ↑ stability
	Indicators	Measurement and assessment tools	Indicators	Measurement and assessment tools	Indicators	Measurement and assessment tools	
	Support environment, policies, and infrastructure	NIH Director Office (RPPR), contract awards, STAR, HCFICs, <i>Tool link</i>	Separation center activity (i.e., Biotech, Pharma)	FDA approvals, patents, industry reports (FDA), Biotech, etc. (BioPharma)	Opinion and research technological innovation	<i>Tool link</i>	
Science							
Public Health							
Broader Societal Impacts							

Types of Value	OUTPUTS			OUT-COMES
	Short-term DISCOVERY	Medium-term VALIDATION	Long-term CONSENSUS	
Science	Reference abstract presentation	Patents	(Linking publications to patents); RPPRs; FDA Orange Book; iEdison	Fundamental Scientific Knowledge
	Primary research articles	Licenses	USPTO database; FDA Orange Book; iEdison	
	Research database and repositories (i.e., NCBI, NDA)	IP transfer agreements (MTAs)	Individual University Admin. Databases (do any aggregate databases exist?)	
Public Health	Development of research resources and infrastructure	Development and initial clinical testing of dx, tx, and px interventions (e.g., FDA applications and Phase I, II, and III clinical trials)	Clinicaltrials.gov; RePORTER (linking projects to clinicaltrials.gov); ExPORTER, RPPRs, NME and IND applications in FDA Orange Book; Comprehensive tools?	Living longer, healthier lives
	Research training reports (e.g., NIH, NCI, NIDA)	Standardized research protocols	Publications databases, but no comprehensive tools	
	Research training reports (e.g., NIH, NCI, NIDA)	Validated data repositories (i.e., NCBI, GenBank, PDB)	Example: NCBI analytical tools; Individual metrics but no comprehensive tools	
Broader Societal Impacts	Advanced testing of evidence-based diagnostic	Drug to market	FDA approvals; Industry Reports	Scientific literacy public ↓ Healthcare-related cost ↑ Productivity ↑ Capacity for Innovation ↑ Global R&D competitiveness Sci. diplomacy ↑ stability
	Advanced testing of evidence-based therapeutic intervention	Dissemination and implementation of practice guidelines, etc.	HHS dissemination and implementation activities; Clinicaltrials.gov; Public health agency recommendations; Publications databases; Comprehensive tools?; Data repository	
	Advanced testing of evidence-based preventive intervention and strategies	Adoption of evidence-based dx, tx, and px practice	Healthcare claims data (i.e., Medicare, Medicaid); HHS; NME and IND applications in FDA Orange Book; Comprehensive tools?	
	Identification of major public health issues and disease risk factors	Public health response	HHS activities (e.g., CDC); NCI activities; Comprehensive tools?	
	Support, expansion, and maintenance of infrastructure	Severe acute respiratory activity (i.e., H1N1, Flu, etc.)	FDA approvals; Grants; Industry reports (RPPRs); Data repository (iEdison); Data repository (iEdison)	
	Create demand for R&D supplies	Enhance STEM education	NSF Reports on Science and Engineering Indicators; NSF, DOE	
	International collaboration	Communication and dissemination of public sector and top-down	IC; Publications; RPPRs; NCI; Publications; CDC (NCHS)	
	Support academic	International of SRT capacity building	Comprehensive tools?	
	Develop pre-competitive response	Support local economy	Comprehensive tools?	
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	Research training reports (e.g., NIH, NCI, NIDA)	Development and initial clinical testing of dx, tx, and px interventions (e.g., FDA applications and Phase I, II, and III clinical trials)	Clinicaltrials.gov; RePORTER (linking projects to clinicaltrials.gov); ExPORTER, RPPRs, NME and IND applications in FDA Orange Book; Comprehensive tools?	
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	Conference abstract/presentation	NIR Research Performance Progress Reports (RPPRs), <i>Task Vehicle</i>	Patent	USPTO database, SPA in RPPORTER, (clinical publications to patents) RPPRC, FDA Orange Book, E-IR	FDA approval of New Drug Application (NDA)		
	Primary research article	PubMed, SCOPUS, ISI, SPA, SPIRES, RPPORTER, RPPR, etc.	License	USPTO database, FDA Orange Book, E-IR	Consensus Development Conference or Briefing (i.e., National Toxicology Program)		
Public Health	Indicators	Measurement and assessment tools	Indicators	Measurement and assessment tools	Indicators	Measurement and assessment tools	Living longer, healthier lives
	Advanced testing of evidence-based diagnostic	RPPR (real research), product, platform, etc., <i>Task Vehicle</i> (diagnostic), <i>Task Vehicle</i> (prevention)	Drug market	FDA approval, Industry Report	Burden of Disease/Quality of Life		
	Advanced testing of evidence-based therapeutic intervention	Clinical trial (pre-Phase II trials), RPPORTER (diagnostic products to clinical trial), E-PORTER, RPPR, FDA Orange Book (approval of NDAs), <i>Task Vehicle</i>	Dissemination and implementation effort, Practice guidelines, etc.	HHS domain-specific RPPR, Clinical trial, recommendation, research, <i>Task Vehicle</i>			
Broader Societal Impacts	Indicators	Measurement and assessment tools	Indicators	Measurement and assessment tools	Indicators	Measurement and assessment tools	Scientific literacy public ↓ Healthcare-related cost ↑ Productivity ↑ Capacity for innovation ↑ Global R&D competitiveness Sci. diplomacy, ↑ stability
	Support government, science, and technology policy	NIR Budget Office, RPPR, contract modeling, STAR METRICS, <i>Task Vehicle</i>	Event prioritization activity (i.e., Biotech, Pharma)	FDA approval, (PATRIOT) Base			
	Create demand for R&D supplier	Purchase research, RPPR, STAR METRICS, <i>Task Vehicle</i>	Enhance STEM education	NIR Report on Indicator, NIS			

Charge Element 2: Methods & Strategies

Existing Metrics: Room to Improve

Gaps

Weaknesses

- 18 - VOBR WORKING GROUP - June 4, 2013

*Charge Element 3: Study Selection***Study Question & Topic Selection**

What topics best communicate and represent NIH's value?

The broad scope of NIH research and the multitude of potential outcomes to be measured pose challenges to assessment efforts. The SMRB is tasked with advising NIH regarding the selection of study topics that are feasible and representative.

- 19 -

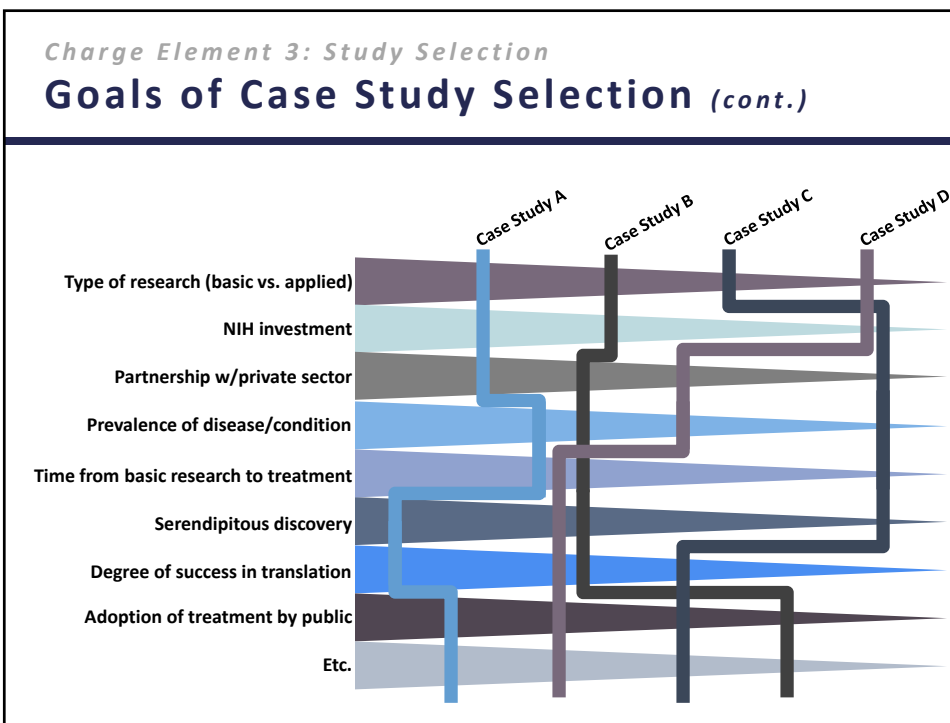
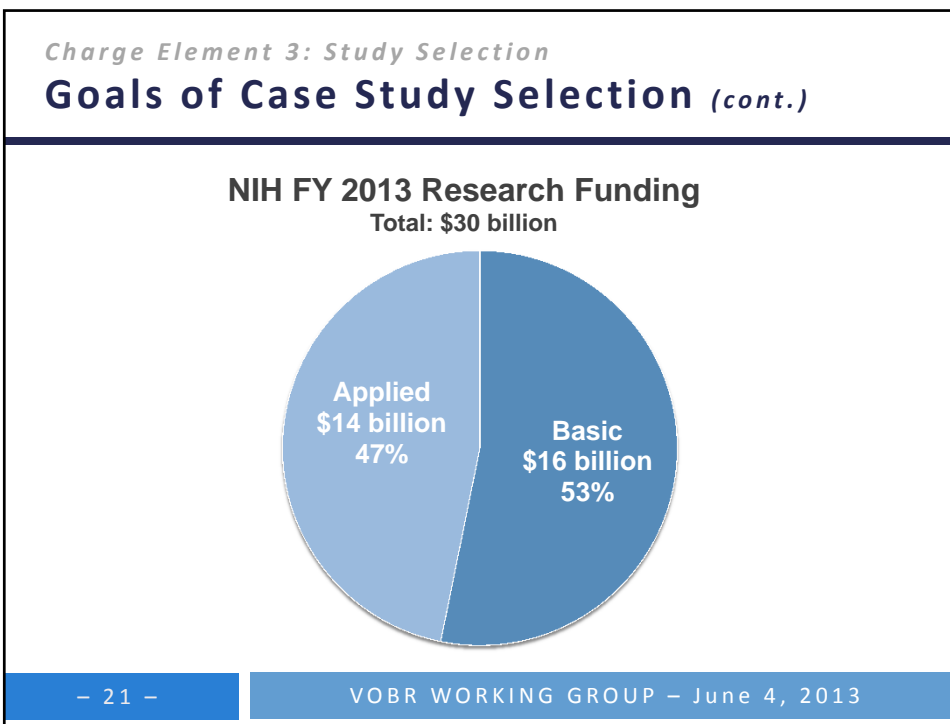
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*Charge Element 3: Study Selection***Goals of Case Study Selection**

- **Illustrate the full spectrum of NIH research, including:**
 - Basic and clinical research
 - Slow and quick time to payoff
 - Successes and “failures”
- **Underscore the importance of investments in basic research**

- 20 -

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Goals for Today's Presentations

- Discuss principles and attributes of how to define and assess value
- Learn of opportunities to improve assessments
- Engage experts in ways to assess value of scientific knowledge, public health, and broader societal effects of biomedical research, including:
 - Strengths and gaps of prior studies
 - Landscape of current efforts
 - Outlook for future endeavors
- Discuss the relevance of these findings to NIH

– 23 –

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Panel Sessions and Roundtable

- **Panel I:** *Assessing the Value of Biomedical Research: Principles, Metrics, Strategies, and Caveats*
- **Panel II:** *Public Health Outputs and Outcomes of Biomedical Research*
- **Panel III:** *Broader Societal Impacts of Biomedical Research*
- **Roundtable Discussion of Value of Biomedical Research Themes**

– 24 –

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Panel Sessions and VOBR Working Group Deliverables			
DELIVERABLES			
	Principles and Caveats	Assessment Methods and Strategies	Study Questions and Study Topic Selection
TYPES OF VALUE	Scientific	Panel I	
	Public Health	Panel II	
	Societal Impacts	Panel III	
Roundtable			