Benefit Cost-Analysis in the Outcome Evaluation of Small Biomedical Research Portfolios

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Introduction

Public funding of research includes many discrete components
• Setting research priorities
• Securing funds
• Funding research infrastructure
• Selecting and funding meritorious projects
• Conducting research
• Monitoring research progress
• Communicating research findings
• Training researchers

Evaluation of research, particularly biomedical research, has entered a period of intense demand for rigorous methods and actionable results. Challenges and opportunities exist in meeting this demand.
Goals of the National Institutes of Health

• Foster fundamental creative discoveries, innovative research strategies, and their applications as a basis for ultimately protecting and improving health

• Develop, maintain, and renew scientific human and physical resources that will ensure the Nation's capability to prevent disease

• Expand the knowledge base in medical and associated sciences in order to enhance the Nation's economic well-being and ensure a continued high return on the public investment in research

• Exemplify and promote the highest level of scientific integrity, public accountability, and social responsibility in the conduct of science
NIH Studies of Economic Return on Investment


$3.8 billion in extramural funding (nominal dollars)

Major findings

• The HGP and “associated research and industry activity directly and indirectly generated $796 billion in U.S. economic output” (p.ES-2)
• Return on $1 investment: $141
• Updated in 2013, greater ROI
• Restated some analyses, smaller ROI
NIH Studies of Economic ROI

Economic Return from the Women’s Health Initiative (WHI) Estrogen Plus Progestin Clinical Trial, Roth et al., 2014

Major Findings

• $260 mil in extramural funding ($ 2012)
• Estimated savings from treatment & illnesses averted
• $35.2 billion saved in direct medical costs
• Net economic return: $37 billion
• Return on $1 investment: $140
University of Michigan
School of Public Health

“What does it take to change the world?”

Example 1

• $5 – Average cost of one malaria bed net
• $12 billion – Estimated direct global costs of malaria (illness, treatment, premature death)

Example 2

• $3.3 million – Cost of 5 year federally funded study on Medicare payments for dialysis, medications, tests
• $225 million – Annual savings to Medicare from implementation of new dialysis payment system based on study
Information Needed to Determine Economic Outcomes of Biomedical Research on Observed Changes in Medical Treatment

• Research associated with the observed change in medical treatment

• Observed change in medical treatment

• Attribution (descriptive or quantified) of the causal effect of observed biomedical research findings on the observed change in treatment
Information Needed to Determine Economic Outcomes

• Research associated with the observed change in medical treatment
  – Purpose and methods, showing at least a clear theoretical or hypothetical link to the treatment of interest
  – Outcomes, showing a clear potential link to the observed change in treatment
  – Costs

• Observed change in medical treatment
  – Description of the initial treatment and the change in treatment
  – Utilization data in population(s) of interest (frequency and intensity) for initial treatment
  – Costs of initial treatment
  – Economic and non-economic outcomes observed or predicted by the change in treatment
  – Identification of target populations most likely to be affected by the change in treatment

• Attribution (descriptive or quantified) of the causal effect of observed biomedical research findings on the observed change in treatment
  – Dissemination of the research findings must be established before a claim of attribution may be made
  – Attribution may be assessed by face validity, temporal validity, formal causal analysis, or other empirical method
Research Methods

• Five research programs identified: dental sealants, early childhood caries, oral cavity & pharyngeal cancers in the elderly, validated cell lines in head and neck cancers, and oral HPV infection
  – Evidence of benefit available in peer-reviewed science literature

• Data collected, consistent with model

• Evaluability assessment conducted: feasibility of economic outcome evaluation, given data collected
  – Recommendations if not feasible
# Research Data Collected

<table>
<thead>
<tr>
<th>Needed element</th>
<th>Available element(s)</th>
<th>Source</th>
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<tbody>
<tr>
<td>Purpose</td>
<td>Proposal, annual reports (narrative)</td>
<td>Internal documents</td>
</tr>
<tr>
<td>Method</td>
<td>Proposal, annual reports (narrative)</td>
<td>Internal documents</td>
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<tr>
<td>Outcomes</td>
<td>Annual reports, publications, patents, policies, treatment guidelines (narrative, quantitative)</td>
<td>Internal documents, public databases</td>
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<tr>
<td>Costs</td>
<td>Extramural program budgets (annual $)</td>
<td>Internal documents, public databases</td>
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## Treatment Change Data Collected

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<tr>
<td>Description, initial</td>
<td>Utilization statistics for dental sealants with subgroup analyses</td>
<td>MEPS, NHANES, internal document</td>
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<td>Description, change</td>
<td>Increases in sealant use, especially in Medicaid populations, 1990-2010</td>
<td>MEPS, NHANES, internal document</td>
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<tr>
<td>Treatment cost</td>
<td>$11.60</td>
<td>Peer reviewed publication</td>
</tr>
<tr>
<td>Economic outcomes claimed (observed or hypothesized)</td>
<td>Benefit &gt; $11.60 for high risk children 7-12 years old, peaking at $15.21 for 9 year olds</td>
<td>Peer reviewed publication</td>
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<tr>
<td>Target population(s) identified</td>
<td>High risk for dental caries</td>
<td>Peer reviewed publication</td>
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## Attribution Data Collected

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<td>Dissemination mechanism identified</td>
<td>Yes, Publication and citation counts</td>
<td>Bibliometric analysis</td>
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<tr>
<td>Face validity</td>
<td>Yes</td>
<td>Expert opinion</td>
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<td>Temporal validity</td>
<td>Yes</td>
<td>Internal portfolio analysis</td>
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<tr>
<td>Causal analysis</td>
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</table>
Results of the Evaluability Assessment

• Was the economic claim of treatment benefit credible?
  – Yes, but comprehensive lit review recommended

• Did utilization data support a treatment change in a targeted population?
  – Yes

• Was the research relevant to observed treatment change?
  – Yes

• Were the research findings disseminated prior to the observed treatment change?
  – Yes

• Was attribution (causal link between research & treatment change) established?
  – No, formal causal analysis (expert panel review) recommended
Conclusion

• Data were available to conduct an economic outcome study on the returns to investment of a federally funded dental sealant research program

• Evaluability assessment supported the need for additional data to:
  – Establish the causal link between the research and the observed change in treatment
  – Estimate the economic benefit of the observed change in oral health treatment

• Based on currently available data, the research program is not ready for the evaluation of economic returns on investment
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• The views expressed in this paper are those of the authors and not necessarily those of the National Institutes of Health or George Washington University.
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