Evaluating High Risk, High Reward Research Programs at the National Institutes of Health:

National Institutes of Health's Director's Pioneer Award (NDPA) and New Innovator Award (NIA)

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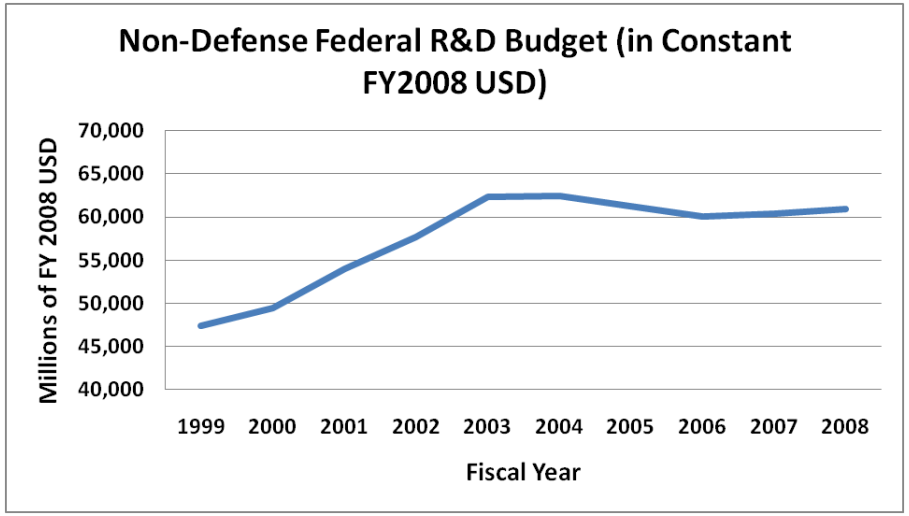
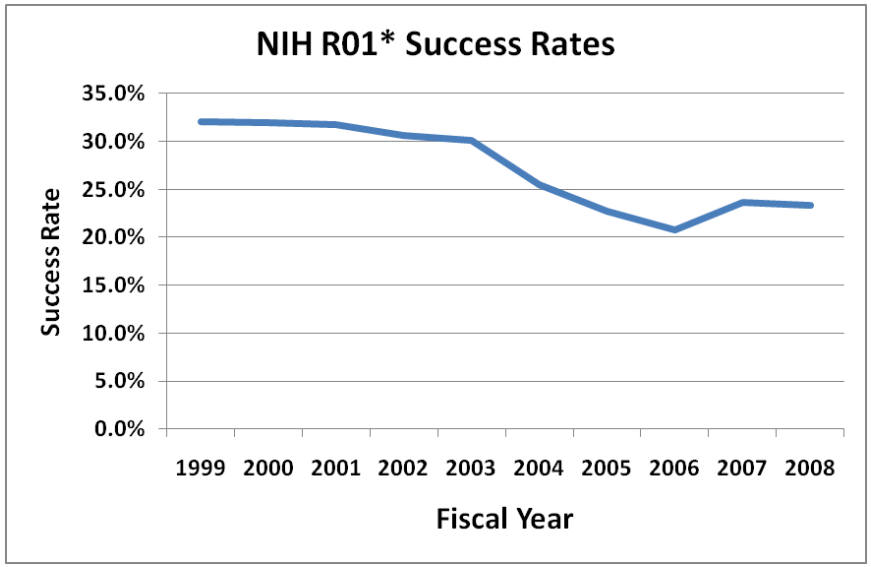
Overview

- The Context
  - What are high risk, high reward research programs and why do we need them?
  - Why do we need to evaluate high risk, high reward research programs?
- Outcome Evaluation of the NIH Director's Pioneer Award
  - The NDPA program
  - Challenges
  - Approach
- Feasibility Study of an Outcome Evaluation of the NIH Director's New Innovator Award
- Closing Thoughts

The Context: Constrained Funds



Source: AAAS Historical R&D Funding Data
http://www.aaas.org/spp/rd/hist09p2.pdf



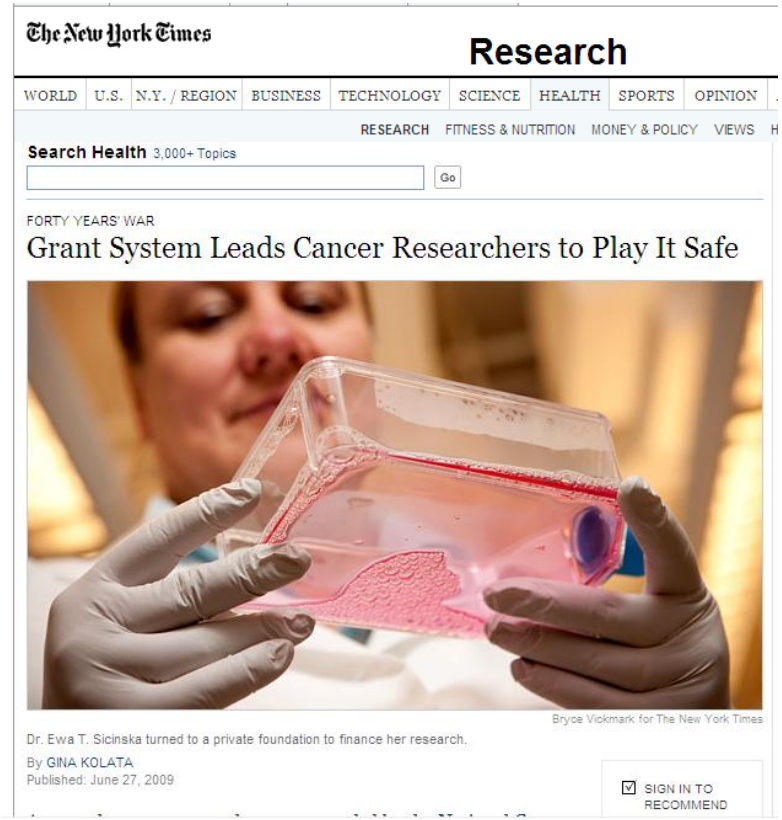
***R01 and R01 equivalent (R23, R29, R37)**
Source: NIH Funding Data
http://report.nih.gov/success_rates/index.aspx

Non-defense R&D budget has stayed relatively flat in recent years, while number of researchers (and grant applications) continues to grow – leading to lowered success rates…

Effects of Budget Constraints – Conservative Science

Funding system has the effect that biomedical researchers "propose" research that's already been done to increase the chances of having their grants funded and to receive follow-on funding…

"And the decision practice of funding agencies is that I only get grants where I have already done some work. See, that's the 'catch 22': I would need external funding to start something new, but since I haven't started the new thing, I can't get external funding." (Laudel, 2006)


The New York Times

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FORTY YEARS' WAR

Grant System Leads Cancer Researchers to Play It Safe

Bryce Vickmark for The New York Times

Dr. Ewa T. Sicinska turned to a private foundation to finance her research.

By GINA KOLATA

Published: June 27, 2009

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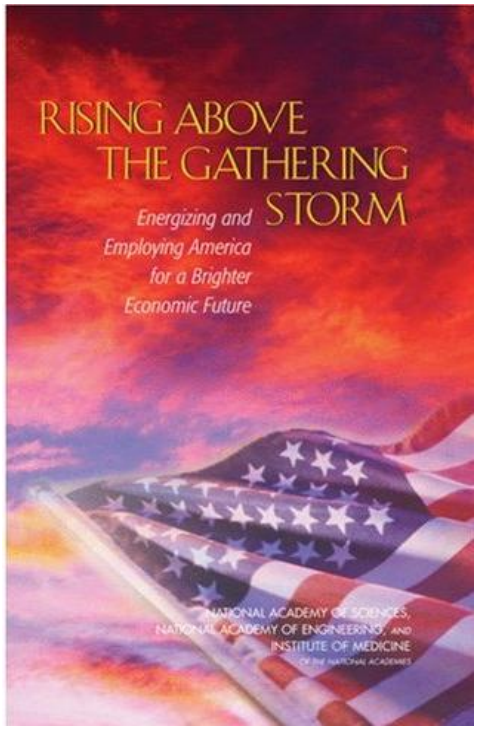
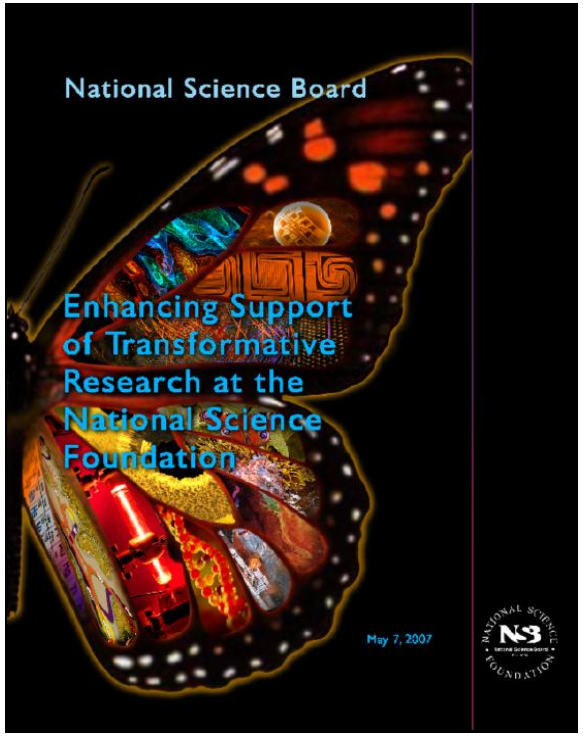
Gerald Rubin: Science far too conservative

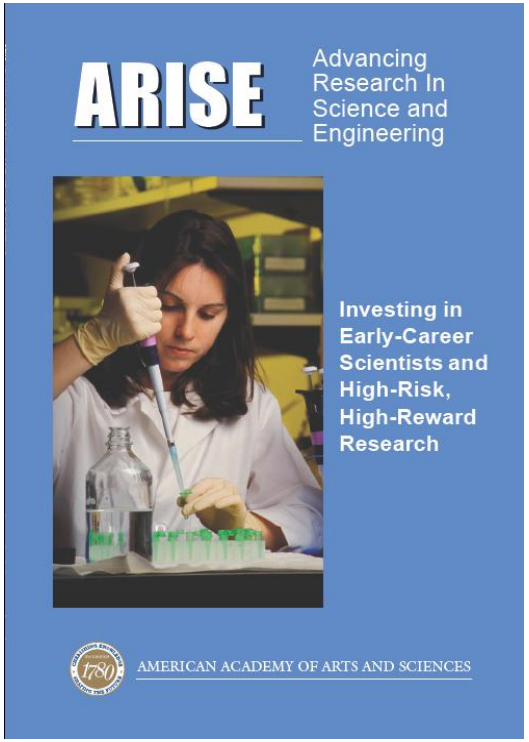
April 20th, 2006

Howard Hughes Medical Institute Director Gerald Rubin says academic and industrial research models have become far too conservative.

The Response – Call for High Risk, High-Reward Research Programs







High-Risk, High-Reward Research Programs in the Federal Government

IDA STPI

Recent blossoming of high-risk, high-reward research programs…

M-09-27

MEMORANDUM FOR THE HEADS OF EXECUTIVE DEPARTMENTS AND AGENCIES

FROM: Peter R. Orszag

SUBJECT: many practical constraints and opportunities that can arise when turning ideas into reality. Agencies should pursue transformational solutions to the Nation's practical challenges, and budget submissions should therefore explain how agencies will provide support for long-term, visionary thinkers proposing high-risk, high-payoff research.

…and likely more to come!

A review of existing programs reveals categories (Lal & Hughes, 2009):

Programs aimed at funding projects based on a technological challenge or critical national need, with strong focus on project management – *"Challenge Programs"*

Programs aimed at funding an individual scientist to undertake (almost) any research project – *"People Programs"*

Programs aimed to move a field forward through teams or interdisciplinary approach – *"Synergy Programs"*

Programs aimed to jump start a project – *"Seed Programs"*

Managing and Evaluating High-Risk, High-Reward Research Programs

IDA STPI

As a community we have little understanding of how to best identify priority areas for funding through these mechanisms, nor how to manage and evaluate these programs

- How risky is "high risk" – how often do we expect projects to fail?
- And how "high" is "high reward" – and are these rewards different than normal science?
- How long will it take to see these rewards?

But as proper stewards of taxpayer money, have a requirement to make sure funds are spent wisely

- Are these programs more effective than "normal" R&D programs?
- Is this type of research better funded by private foundations?

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NDPA Program

NIH DIRECTOR'S

PIONEER

A·W·A·R·D

Initiated in 2004 as pilot program of the NIH Roadmap for Medical Research

"History suggests that leaps in knowledge frequently result from exceptional minds willing and able to explore ideas that were considered risky at their inception, especially in the absence of strong supportive data. Such individuals are more likely to take such risks when they are assured of adequate funds for a sufficient period of time, and with the freedom to set their own research agenda." [Program Notice]

→*person-based*
→ *risky science*
→ *not a continuation of existing research*
→ *more money than an R01, over a longer period of time – but no renewal funds*
→ *extreme flexibility in how funds are used*

NDPA Outcome Evaluation: Challenges

- Program flexibility in definition of pioneering – relies on experts' view of "you know it when you see it"
  - Literature also shows a lack of (ex-ante or short-term) quantitative measures of pioneering research
- Uniqueness of program selection processes
  - Heterogeneity of awardees
- Timing of evaluation – only 5 years after awards made
- Program evolution over the five years
- Small number of awardees (~10/year)
- Flexibility in how funds are used

NDPA Outcome Evaluation: Approach

- Phased approach
  - Phase I: Feasibility Study
  - Phase II: Near-term outcomes of first two cohorts
  - Phase III: Longer-term outcomes; near-term outcomes of later cohorts

- Pre-design activities:
  - Literature Review
  - Interviews with Stakeholders (NDPA External Advisory Committee, NDPA Program Officials)
  - Focus group with a sample of NDPA awardees
  - Interviews with evaluation and creativity specialists

Study Questions

Are NDPA awardees doing pioneering research?

What are the spillover effects of the NDPA program on…

- The awardees, their students and their institutions?
- The NIH?
- The scientific community?

Mixed-Method Approach

- Case Studies (Yin, 2002, GAO, 1990) of all 22 awardees from first two years of program
  - In-depth interviews with pioneers, analysis of applications and progress reports
  - Site visits, surveys of lab members
  - Quantitative and qualitative analysis of publications and patents, including social network analysis

- Expert Panel review (NAP, 1999, SoSP ITG, 2008)
  - Review of case study packets
  - Pre-discussion Survey + Cognitive Interviews (Beatty, 2004), Expert Discussion, Post-discussion Survey + Cognitive Interviews

Operationalizing Pioneering?

Pioneering Research Activities (Colwell, 2007)

- Fundamental ideas underlying research were at odds with prevailing wisdom
- Research required use of equipment or techniques that have not been proven or are extraordinarily difficult
- Research required knowledge of fields beyond previously demonstrated area of expertise
- Research involved a unique and unprecedented combination of perspectives, disciplines, or approaches

Pioneering Research Outcomes (Heinze & Shapira, 2007)

- Research could result in the formulation of a novel idea (or set of ideas) that could instigate a new cognitive frame or advance theories to a new level of sophistication.
- Research could result in the discovery of new empirical phenomena that could stimulate the generation of new theories.
- Research could result in the development of a new methodology, enabling empirical testing of theoretical problems.
- Research could result in the invention of novel instruments that could instigate new search perspectives and research domains.
- Research could result in new integration of formerly disparate ideas into general theoretical laws enabling analyses of diverse phenomena within a common cognitive frame.

Approach: Pros

- Case study approach allows for discovery of aspects of pioneering research not anticipated
- Triangulation and complentarity by using multiple methods increases validity
- Operationalization of “pioneering” (activities and outcomes) and its relation to terms such as:

Interdisciplinary	Risky	Useful
Creative	Novel	Relevant to
Innovative	Transformative	Human Health

- Phased approach allows for baselines for assessments of longer term outcomes

Approach: Cons

- Resource-intensive: feasible for 22 awardees, but impractical for larger samples
- Issues associated with Expert Panel – biases and conflict of interests (Langfeldt, 2002)
- Comparison group would be ideal – and plan on one in next phase

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NIA Program

NIH DIRECTOR'S NEW INNOVATOR AWARD

- Launched in 2007, also as part of the Roadmap
- Often called "junior NDPA" – PIs must be <10 years from last degree; no previous R01 funding
- NIA has two goals:

(1)stimulating creative research

(2)supporting new investigators

NIA Outcome Evaluation: Challenges & Approach

- Timeliness of evaluation – only 5 years after awards made
- Uniqueness of program selection processes
- Small-ish number of awardees (~30/year)
- Flexibility in how funds are used

- Quantitative approach
- Comparison group: non-awardees (finalists) matched on variables using Propensity Score Matching (Pion and Cordray, 2008, Azoulay et al, 2009)

Results…Stay Tuned!

Progress:

- Developed Pioneer and Lab Member interview protocol
- Piloted interview protocol (~~awaiting OMB clearance to proceed~~)
- Developing Expert Panel discussion guide
- Performing Bibliometric Analyses

Initial Findings:

- NDPA has enabled awardees to quickly move resources based on unexpected findings, either within their lab or beyond
- NDPA has enabled work to be done on complex, resource (both time and money)-intensive work, such as screenings or technology development
- NDPA-proposed work was either accomplished within the first few years of the award or is expected to take much longer than the 5 years
- Lag-time between award and publications on average 3 years

Closing Thoughts

- Challenge for us as evaluators to be pioneering in our approaches and to be sensitive to the nature of these programs in our design, implementation, and dissemination of findings
- There's a need for cross-evaluation comparisons and meta-evaluations
- Encourage engagement of Evaluation community with the Science of Science Policy community

Acknowledgements

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Questions?

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