Evaluation of the NIH Director’s Pioneer Award Program-DP1

May 14, 2013

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Current Common Fund Programs

Pioneers
New Innovators
Transformative Research Awards
Early Independence Awards

PROMIS: Clinical Outcomes Assessment
Regulatory Science
NIH Medical Research Scholars
NIH Center for Regenerative Medicine
Gulf Oil Spill Long Term Follow Up
Protein Capture
Technology Centers for Networks and Pathways
Nanomedicine
Knockout Mouse Phenotyping
Bioinformatics and Computational Biology
Global Health
Metabolomics
Single Cell Analysis

Pioneers: New Innovators: Transformative Research Awards: Early Independence Awards

Interdisciplinary Research Consortia
Undiagnosed Diseases Program
Clinical Science and Translation Awards (CTSAs)
High-Risk Research
HCS Research Collaboratory
Health Economics
Bridging Interventional Development Gaps (BrIDGs)

NIH Common Fund

http://commonfund.nih.gov/
Origins of the NIH Director’s Pioneer Award Program

- Begun in 2004 as one of the first programs of the NIH Roadmap

- Initiated to address concerns that high risk, visionary research was not being supported due to the conservative nature of existing NIH funding mechanisms.

- Based on the premise that “Person Based” application and review processes would reward past creativity and encourage innovators to go in new directions

- Research to be conducted must represent a substantial departure from the work that the investigator (or anyone else) has done in the past: PIONEERING RESEARCH.

- Experiment in science management with a new mechanism
The DP1 Mechanism

- **Target:** Creative individuals proposing paradigm shifting research
- **Eligibility:** Open to all career stages
- **Prelim Data:** Not required; may be included
- **Project Description:** 5 page essay
- **Ref letters:** 3 letters required
- **Effort:** Minimum of 51%
- **Budget:** None submitted, $500k/yr for 5 years
- **Review:** Multi-phased, Panel interview of finalists
Assessment of the Program

• The Pioneer program is cited as an example of successful government investment in innovation.
• Several awardees have done spectacular work, creating new fields of research.
• BUT:
  – R01 investigators have also done spectacular work, creating new fields of research. *Is the DP1 award mechanism/review process better at identifying and supporting innovative, high impact research?*
    • If similar investigators applied for and received an R01, would they do just as innovative/high impact research?
    • If you have $1M to spend, will you get more innovation/impact with DP1s or R01s?
Assessment of the Program (cont)

• Pioneer Awards emphasize past performance of the PI in the review more than is typical for R01s. How do Pioneer Awardees compare to other investigators funded based on past performance?
  – HHMI Investigators offer certain similarities in this respect. However:
    • Research via HHMI is not restricted to new directions.
    • HHMI provides support to the lab as a whole – not a given project.
    • HHMI awards are renewable; long term risky ventures may seem more appealing.
Assessment of the Program (cont)

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A formal evaluation

- To address these questions, the Institute for Defense Analyses - Science and Technology Policy Institute (STPI) was commissioned to undertake a comparative evaluation of the Pioneer Program.
  - This briefing presents selected findings. See full report for complete analyses.
- Pioneer awardees from 2004, 2005, and 2006 were included, since their awards have ended. (35)
- 4 Comparison Groups were chosen:
  - Matched R01s
  - Random funding–matched R01s
  - HHMI Investigators from 2005 (39)
  - nonfunded Pioneer finalists
Matched R01s: How does a DP1 compare to an R01?

• To answer this question, you need to have a similar set of investigators with the two different grant mechanisms.

• R01s with similar characteristics as Pioneer Awards
  – Awarded during the same period
  – Involved in similar areas of science
  – PIs at similar career stage, with similar backgrounds
  – PIs at similar institutions

• This produced a list of R01s with a combined budget of about 50% that of the Pioneers
Random R01 Portfolios: If you have $87.5M to spend, will DP1s or R01s buy more innovation/impact?

• Pioneers from 2004, 2005, and 2006 collectively cost $87.5M, involving 35 awards
• How did these awards compare to sets of R01s that consumed the same budget?
  – 30 random trans-NIH portfolios of R01s were selected with similar total costs
  – Award number per portfolio ranged from 64-100
HHMI Investigators: Does the Pioneer program do a similar job of fostering innovation as HHMI?

• The HHMI program purpose states that:
  “by appointing scientists as Hughes investigators, rather than awarding them grants for specific research projects, the investigators are provided with long-term flexible funding that gives them the freedom to explore and if necessary, to change direction in their research. Moreover, they have support to follow their ideas through to fruition – even if that process takes a very long time.”

• HHMI investigators from 2005 competition were nominated by presidents and deans of the top 200 NIH-funded institutions

• Direct costs of $600K per year plus salary and equipment costs are provided

• Reappointment rate at the end of 5 years is 80%, and investigators usually stay with HHMI for an average of 15 years.
## Summary of Comparison Groups

<table>
<thead>
<tr>
<th>Comparison Group</th>
<th>Description</th>
<th>Research Question</th>
<th>Advantages</th>
<th>Limitations</th>
</tr>
</thead>
<tbody>
<tr>
<td>Matched R01s N = 35</td>
<td>R01s matched on PI characteristics within similar research areas</td>
<td>To what extent do Pioneer award outcomes have more (or less) impact as compared with those of traditional NIH grants given to similarly qualified researchers?</td>
<td>Controls for PI-related characteristics that may impact outcome</td>
<td>Does not control for award size</td>
</tr>
<tr>
<td>30 Random R01 Portfolios N_av = 85</td>
<td>30 randomly selected portfolios with portfolio direct costs comparable to that of the NDPA</td>
<td>To what extent do the outcomes of the NDPA portfolio have more (or less) impact as compared with 30 similarly sized portfolios of R01?</td>
<td>Controls for portfolio size ($)</td>
<td>Portfolios contain different numbers of grants; Does not control for PI characteristics</td>
</tr>
<tr>
<td>HHMI N = 39</td>
<td>2005 Howard Hughes Medical Institute Investigators</td>
<td>To what extent do Pioneer award outcomes have more (or less) impact as compared with a similarly high-prestige research program?</td>
<td>High prestige program that funds high risk high reward research in a way that is similar to NDPA in many aspects; reputation for innovative investigators</td>
<td>Does not explicitly control for PI characteristics or award size</td>
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</tbody>
</table>
Multi-Method Approach for Comparison Analysis

- **Bibliometrics**
  - Analyzed over 20,000 publications
  - Other descriptive analyses

- **Expert review**
  - 94 experts conducted over 1,500 reviews
    - Mechanism of funding blinded in papers’ Acknowledgements sections
  - Analyzed ratings
    - Impact of researchers’ “top-5” publications
    - Innovativeness of the approaches in the researchers’ “top-5” publications
  - Analyzed qualitative comments

<table>
<thead>
<tr>
<th>Publications</th>
<th>Citations</th>
<th>Journal Impact</th>
<th>Expert Assessments</th>
</tr>
</thead>
<tbody>
<tr>
<td>Counts</td>
<td>Counts per $</td>
<td>Counts per Grant</td>
<td>Top Journals</td>
</tr>
<tr>
<td>Counts per Grant</td>
<td>Counts per Paper</td>
<td>Per $</td>
<td>Packet of 5 top papers</td>
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<tr>
<td>Per $</td>
<td>H Index*</td>
<td>JIF</td>
<td>Individual Paper</td>
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* An index that captures both the productivity of and citations to published work

**NOT ALL METRICS CAN BE USED FOR ALL COMPARISON GROUPS**
Expert Review: Impact

• Extremely high impact research accomplishes 1 or more of the following:
  – Radically changes present understanding of an important existing scientific or engineering concept
  – Leads to the creation of a new paradigm
  – Challenges present understanding
  – Provides pathways to new frontiers
  – Challenges conventional wisdom
  – Leads to unexpected insights that enable new technologies or methodologies
  – Redefines the boundaries of science
Expert Review: Innovativeness of Research Approaches

• “Extremely Innovative” research accomplishes one or more of the following:
  – Ideas are at odds with prevailing wisdom
  – Research requires the use of equipment or techniques that have not been proven or are considered extraordinarily difficult
  – Research involves a unique combination of disciplines
Results: DP1 vs Matched R01
please see the full report
DP1s produce a greater number of publications per grant, but the same number per dollar as compared with matched R01s.

Note: Data from NIAID eSPA and Web of Science for all groups

Kolmogorov-Smirnov p = .02, 0.47
DP1 publications appear in journals with a higher journal impact factor, and awards have a higher H-index rating.

Kolmogorov-Smirnov p < 0.0001, 0.003
However, NDPA publications have a longer “tail” of citations.

Kolmogorov-Smirnov p = .34
Experts assess DP1 research as having more impact and innovation.

- Impact:
  - NDPA Awardees
  - Matched R01 PIs

- Innovation:
  - NDPA Awardees
  - Matched R01 PIs

**Statistical Tests:**
- t-test, \( p < 0.0001 \), K-S, \( p = 0.0002 \)
- t-test, \( p < 0.0001 \), K-S, \( p = 0.0005 \)
Pioneer PORTFOLIO
VS
Random R01 PORTFOLIOS

per $ comparison summary at the end
Pioneers vs HHMI
HHMI investigators publish more papers, and the have more total citations……

Kolmogorov-Smirnov $p = 0.79, 0.02$

Kolmogorov-Smirnov $p = 0.18, 0.04$
....but the publications and citations per $ is about the same.

NDPA alone ≈ $600k
HHMI alone ≈ $960k

Kolmogorov-Smirnov p = 0.68, 0.32
Pioneers Publish in Journals with Lower Impact Factors than HHMI

Kolmogorov-Smirnov p < 0.0001, < 0.0001
DP1 PIs have the same H-index in 2011 as HHMI Investigators

Kolmogorov-Smirnov $p = 0.26, 0.69$
Experts assess Pioneer research as having similar impact and innovation as HHMI.

**Graphical Representation:**
- **Impact:**
  - NDPA Awardees
  - Matched R01 PIs
  - HHMI

- **Innovation:**
  - NDPA Awardees
  - Matched R01 PIs
  - HHMI

**Statistical Tests:**
- **DP1: R01**
  - T-test, $p < 0.0001$, K-S, $p = 0.0002$
  - T-test, $p < 0.0001$, K-S, $p = 0.0005$
- **DP1: HHMI**
  - T-test, $p = 0.08$, K-S, $p = 0.17$
  - T-test, $p = 0.71$, K-S, $p = 0.88$
### Summary of Expert Assessment

<table>
<thead>
<tr>
<th>NDPA does</th>
<th>Matched R01 (Grant Level)</th>
<th>HHMI Investigators</th>
</tr>
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<tbody>
<tr>
<td>Impact</td>
<td></td>
<td></td>
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<tr>
<td>Packets</td>
<td>Green</td>
<td>Green</td>
</tr>
<tr>
<td>Papers</td>
<td>Green</td>
<td>Red</td>
</tr>
<tr>
<td>Innovativeness of Research Approaches</td>
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<tr>
<td>Packets</td>
<td>Green</td>
<td>Green</td>
</tr>
<tr>
<td>Papers</td>
<td>Green</td>
<td>Yellow</td>
</tr>
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Experts were asked to rate PI papers individually and as a set, with the set referred to as a packet. A packet included a researcher’s up-to-five publications with the highest presumed (by the researcher, NIH program officer, or STPI) impact.

*Note:* Colors indicate how the NDPA group rated compared with other groups. For example, green (rated higher) indicates the NDPA group rated higher than the comparison group on a given metric.
## Bibliometric Impact of DP1 Compared with Other Groups

<table>
<thead>
<tr>
<th></th>
<th>Matched R01 (Grant Level)</th>
<th>Random R01 Portfolios</th>
<th>HHMI Investigators</th>
<th>DP1 Finalists</th>
</tr>
</thead>
<tbody>
<tr>
<td>Number of citations per awardee</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Number of citations per grant funding amount</td>
<td></td>
<td></td>
<td></td>
<td>N/A</td>
</tr>
<tr>
<td>Number of citations per publication</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>H-index</td>
<td></td>
<td></td>
<td>N/A</td>
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<tr>
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</table>

DP1 does **better** compared to Matched R01 (Grant Level), **same** compared to Random R01 Portfolios and HHMI Investigators, and **worse** compared to DP1 Finalists.
Possible Reasons for Differences

• It appears that higher funding leads to higher portfolio-level impact.

• DP1 vs matched R01: may be due to differences in funding or program characteristics (such as increased flexibility).

• DP1 vs random R01 portfolios: may be due to differences in PI characteristics, research area, or program characteristics.

• DP1 vs HHMI: likely not attributable to flexibility of research, or riskiness of ideas, but may be due to funding level and stability, differences in PIs, or differences in areas of science.
Going Forward

- This assessment compels us to continue the support for the Pioneer program and to celebrate the trail blazing opportunities it provides.

- Scientific progress results from following many paths, and the different funding mechanisms used by the NIH can each facilitate progress in different ways.

- R01 supported research provides the depth and breadth to the scientific research portfolio which is required to afford meaningful and directed understanding.
QUESTIONS, DISCUSSION
Years Since Degree

![Graph showing box plots for different groups]

- NDPA Awardees
- Matched R01 PIs
- HHMI
- NDPA Finalists
- Random Portfolios

The graph compares the years since degree for different groups, indicating variations in the distribution of years since degree.
Institutional Rankings

Source: Elsevier SCImago