NATIONAL CANCER INSTITUTE

Center for Strategic Scientific Initiatives

THE PROVOCATIVE QUESTIONS INITIATIVE PROGRAM EVALUATION

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EXECUTIVE SUMMARY

Since 2011, the Provocative Questions (PQ) Initiative has provided support for cancer research that addresses important questions that are broadly considered challenging or understudied. The PQ Initiative focuses on asking difficult questions and has been implemented as an effort to solicit new approaches from diverse scientific disciplines. In 2016, an evaluation of the program was performed by Clarivate Analytics (formerly the IP & Science business of Thomson Reuters). This evaluation builds upon a previous evaluation conducted by Clarivate Analytics in 2014, and used both quantitative and qualitative methods to evaluate the PQ Initiative, with a focus on the three following evaluation questions:

1. How effective are PQ program processes?
2. Did the size of PQ research areas increase following the issuance of each PQ?
3. Has the PQ Initiative supported high quality and novel science in the tarted areas?

The key findings of the evaluation are summarized below.

How effective are PQ program processes?

Overall, the PQ program processes were found to be effective. Interviewees generally found the question development process to be democratic and inclusive. In addition, the members of the Executive Committee and the Workshop Participants indicated that workshops were a useful mechanism to develop and select questions, despite the high level of effort required to administer the PQ program. The initiative has successfully targeted research areas that were not well represented in the literature prior to the issuance of a question. The requirement to retire PQs was well received by Applicants and Awardees, and was seen as an important feature of the program that ensures that the most current PQs are the focus of the funding initiative. However, there was disagreement over which specific questions should have been retired. The Reviewers and other stakeholders interviewed recognized that the PQ initiative is a unique mechanism and has distinctive requirements. The majority of both groups recognized the lessened emphasis on preliminary data in the Request for Applications (RFAs) as a strength; however, not all participants in the program were aware of that fact prior to applying or serving as a Reviewer. An evaluation of the scores received by the applications during the review process revealed that the Reviewers took this de-emphasis into account.

Did the size of PQ research areas increase following the issuance of each PQ?

One of the overarching goals of the PQ program is to highlight understudied and important cancer research areas. There was an increase in the estimated total share of cancer research in two thirds of the PQs after each question was incorporated into the program. Additionally, the estimated numbers of authors working in the research areas increased for 85% of the PQs after the questions were introduced. It is important to note that there may be other factors beyond the PQ program that are causative for this increase (such as other funders or organic growth of a research field), particularly as the PQ program funds a small percentage of the publications in most of PQ research areas. An analysis of all publications supported by the National Cancer Institute (NCI), indicated that the majority of PQ research areas are attracting fewer new Principal Investigators (PIs) to NCI than the average NCI R01 and R21 grants, suggesting that these research areas may not be as attractive to researchers starting their independent careers and are not an easy entry point into the cancer research field for investigators that have an alternative research focus.

Has the PQ initiative supported high quality and novel science in the targeted areas?

A preliminary evaluation of the impact of the PQ program was performed. On average, each PQ funded project has produced four publications. Approximately half of these publications were in the research area associated with the PQ through which the project received funding. Since the PQ portfolio intentionally targeted areas with more risk associated with them, one would not expect all publications to be in the

original targeted research area. The normalized citation impact of papers funded by the PQ program is twice as high as the other papers in the PQ research areas. The majority of Awardees (85%) indicated that they had new research findings that directly resulted from PQ funding. In addition, 65% of Awardees had identified new methods or model sets. Branch Chiefs and Program Directors cited promising approaches and early successes. These included work in cachexia; social and neuroscience advances in message processing; the role of positive emotions in physical exercise; and biological aging and colon cancer.

PROGRAM OVERVIEW AND EVALUATION GOALS

This report provides an overview of an evaluation of the National Cancer Institute's (NCI's) Provocative Questions (PQ) Initiative, conducted in 2016 by Clarivate Analytics. In this section of the report, a brief introduction to the PQ program is provided, as well as a description of the three main evaluation questions used to guide the evaluation. Finally, an overview of the evaluation approach is provided.

Program Overview

Initiated in fiscal year (FY) 2011, the PQ Initiative provides support for cancer research that addresses important questions that are broadly considered challenging or understudied. The PQ Initiative complements NCI's broader funding portfolio with a more flexible Request for Application (RFA) design, a focus on asking difficult questions, and an effort to solicit new approaches from diverse scientific disciplines. The PQ Initiative has solicited applications in 2011, 2012, 2013, and 2015 (the most current issuance). At the time of this evaluation, additional applications for the 2015 issuance were still being reviewed; thus, this evaluation only includes the 2011, 2012, and 2013 issuances. In addition, the 2013 issuance was only included in the qualitative component of this evaluation. This issuance was not included in the quantitative component of the evaluation because it was deemed too early to observe any sizable effect of the PQs that were newly implemented in this issuance.

In its first three issuances, the PQ Initiative funded 10.3% of applications submitted (188/1822), for a total of $183.2 million in new awards. The PQ program utilized two funding mechanisms: R21 and R01. To date, the majority of grant applications (58%) and awards (62.2%) have been R01s.

Evaluation Goals

This evaluation focuses on the following three overarching evaluation questions, and when appropriate, considers the impact in the targeted research areas before and after the establishment of the program:

1. Are PQ program processes effective?
2. Did the size of PQ research areas increase following the issuance of each PQ?
3. Has the PQ initiative supported high quality and novel science in the targeted areas?

Quantitative and qualitative results will be provided for each of the evaluation questions above. The report will focus on program wide trends.

Evaluation Method

A mix of literature-based quantitative and feedback-based qualitative evaluation approaches were utilized throughout the evaluation. Feedback was provided throughout the evaluation process by an independent Evaluation Advisory Committee. The evaluation was conducted using methods that included topic modeling, a bibliometric analysis of the relevant literature, as well as interviews and surveys of key stakeholders.

Feedback from various stakeholder groups was solicited in the spring of 2016 using interviews and survey instruments. Interview and survey questions were focused on the PQ development process, application process, review process, management process, quality, and the scientific outcome of PQ research. In addition, the stakeholders were also asked about their perception of community enthusiasm, innovation of the projects and successes of the PQ program. Key stakeholder groups and participants interviewed included Executive Committee members, Branch Chiefs/Program Directors, Reviewers, and Workshop Participants. In addition, an online survey was conducted to solicit feedback from the PQ program Applicants (whose applications were not awarded) and Awardees. The Science and Technology Policy Institute designed

and conducted focus groups with stake holder representatives organized in late 2015 to inform the design of the interview and survey guide. The interview and survey guides were designed by the Madrillon Group and vetted by the Evaluation Advisory Committee. The interviews and surveys were conducted by the Madrillon Group.

A quantitative analysis based on proposal applications, funded publications, and PQ-related literature was also conducted. In this evaluation, an explicit assumption made is that the number of PQ-related publications can be used as a proxy for the size of each PQ research area. The quantitative analysis targeted 33 Provocative Questions (questions for short) from the 2011 and 2012 issuances and looked at literature published between 2008 and 2015. Since it would be impractical to review all scientific publications for inclusion in the evaluation, a mixture of machine learning and subject matter expert (SME) review techniques were employed to identify PQ-related publications. Business rules were developed in a previous evaluation to identify cancer publications between 2008 and 2015. Topic modeling was used as a practical tool to assist NCI SMEs with identification of PQ-related publications from the approximately 363,000 cancer publications identified during this time period. Importantly, the NCI SMEs were relied upon to interpret the nuances of each PQ to isolate PQ-related publications from this candidate set for subsequent analyses. The resultant sizes of PQs in terms of publication count between 2008 and 2015 range from tens to thousands of papers. Project information and reported funded publications were identified and obtained from the NIH RePORTER system. Additional application-specific data such as criterion and overall impact scores of applications were obtained via the Query, View, and Report (QVR) system of NIH.

RECOMMENDATIONS

In this evaluation, the following three evaluation questions were considered:

1. Are PQ program processes effective?
2. Did the size of PQ research areas increase following the issuance of each PQ?
3. Has the PQ initiative supported high quality and novel science in the targeted areas?

Regarding the **PQ program processes**, it was found that the overall PQ program processes were effective in targeting important research areas that are underrepresented in the overall cancer field. Interviewees generally found the question development process to be democratic and inclusive and the workshops were a useful mechanism to develop and select questions, despite a significant amount of effort is required for the current process. While the lessened emphasis on preliminary data was recognized as a positive feature of the program and was taken into account when applications were reviewed not all Applicants and even Awardees were aware of this aspect of the Initiative. Lastly, the Applicants and Awardees were not in agreement over which specific questions should have been retired.

Given these results, it is recommended that the PQ program management:

- Continue to improve and optimize program management processes, including questions development and application review to lessen burdens on participating program officers.
- Provide summary of retirement decisions to improve transparency.
- Clarify the de-emphasis on preliminary data more in future RFAs.
- Continue to track the chronological trend of the publication related to PQ in the next several years to determine whether there is an optimal number of issuances to catalyze risky or understudied research in fields, and whether there is a difference between those PQs viewed as retired too soon or too late.

Regarding the support PQ Initiative provides to **increase the size of PQ targeted research areas**, it was found that all PQs successfully targeted understudied areas and in two thirds of the PQs, there was an increase in the estimated total share of cancer research since the establishment of the PQ Initiative, after correcting for a baseline increase in cancer research. The NCI remained the principal force among NIH ICs in funding research in these areas. While the estimated numbers of authors have also increased, most of the PIs in PQ targeted research areas are not new to NCI.

Given these results, it is recommended that the PQ program management:

- Review the role, experience, and type of researchers best suited to pursue such risky projects and consider strategies to target these candidates.
- If there is a desire to attract new or early career researchers to NCI in the PQ research areas, consider what might be potential barriers to new researchers. For example, an early career researcher may look to apply for traditional R01s where there may be options or renew or re-compete at the end of the maximum funding period. As another example, R21 may not be the best option for early career researchers as it limits the project period to 2 years.
- Investigate methods to identify and target candidate researchers who may be interested in future PQs, especially those not in NCI who are already working in related areas.

Regarding the support the PQ Initiative provides to **support high quality and novel science in the tarted areas**, it was found that PQ-funded projects have been productive and their publications have been cited almost twice as much as other publications in the same field. However, approximately half of their publications were in the research area associated with the PQ through which the project received funding. While we recognize that it may still be early for these project's impact to fully develop and for the community to fully appreciate the results, there are promising successes identified both by the funded investigators and the Program Directors.

Given these results, it is recommended that the PQ program management:

- Consider realistic expectations of research outcome for each PQ for better measurement of impact.
- Revisit the evaluation in five years after there has been sufficient time for the impact of the program to be fully developed and observable.
- Characterize research areas that have high percentage of PQ-related publications to inform PQ development and subsequent program management.
- Evaluate the type of grants and support that are needed by investigators to initiative research in PQ areas after questions are retired.