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Background

The goal of the Roadmap Transformative Research Projects Program (T-R01) is to support “groundbreaking, exceptionally innovative, high risk, original, and/or unconventional research with the potential to create new scientific paradigms or challenge existing ones.” The program was established in 2009 with a budget of $25 million and funded 42 investigators in the first round of competition. Funding for the T-R01 program comes from the NIH common fund.

The second round of applications was due in January of 2010 and the applications are now undergoing peer review. While the intent and budget of the program remained the same, the 2010 RFA no longer included highlighted areas; any research in clinical, basic, behavioral, and translational sciences that meets transformative criteria was eligible for funding.

As in 2009, the applicants were directed to use a specific format, which differed from the traditional R01 submissions both in length and in content. All applications had to include a 2-page Biosketch; an 8-page Research Plan (with subsections: Challenge and Potential Impact, Approach, Appropriateness of T-R01 Mechanism, Timeline); and a 1-page Bibliography and References.

The review process, likewise, remained the same and included three stages. In Stage 1, all proposals were screened by a panel of 12 “generalist” reviewers (the names were known to the applicants), whose role was to evaluate the applications for transformative potential, innovation, and significance. In Stage 2 the applications were evaluated for technical merit by a significantly larger panel of mail reviewers, whose expertise matched the proposed research. Finally in Stage 3, the same Stage 1 reviewers convened in person to make final decisions based on their own preliminary evaluations and on the input from the Stage 2 experts.

Methodology

To assess participant satisfaction with the program, CSR administered web-based surveys to 545 T-R01 applicants, 12 Stage 1/3 reviewers, and 215 Stage 2 reviewers. The surveys were fielded in May of 2010, before the applicants were notified of the status of their application, but after Stage 1/3 reviewers had made final decisions. Response rates were 59% for applicants, 42% for Stage 1/3 reviewers, and 52% for Stage 2 reviewers.

Survey protocols contained multiple-choice and open-ended questions. The applicants were asked about their background and funding history; satisfaction with the application process and materials; and characteristics of transformative research. Questions to reviewers included their views on the clarity of instructions; the review process; and the applicant pool. This report is a summary of survey data.

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1 Roadmap Transformative Research Projects Program (R01). RFA-RM-09-022.
Findings: Applicant Feedback

This section is a summary of information the applicants provided about themselves as well as of their views about T-R01 program.

Demographic information

Of the applicants responding to the survey, 60% were White, 31% Asian, and 2% African American (data not shown). Seventy percent were 36–55 years old (Exhibit 1) and 75% male (data not shown).

Exhibit 1: Applicant age, N=323

<table>
<thead>
<tr>
<th>Age Group</th>
<th>Number</th>
</tr>
</thead>
<tbody>
<tr>
<td>25-35</td>
<td>4</td>
</tr>
<tr>
<td>36-45</td>
<td>32</td>
</tr>
<tr>
<td>46-55</td>
<td>38</td>
</tr>
<tr>
<td>56-65</td>
<td>19</td>
</tr>
<tr>
<td>66-75</td>
<td>5</td>
</tr>
<tr>
<td>76+</td>
<td>1</td>
</tr>
</tbody>
</table>

Scientific focus and funding history

Respondents were asked to classify their research using categories established by NIH. Biological scientists emerged as the most common group (N=255), with clinical scientists a distant second (N=87) and behavioral scientists third (N=39). Note that the total number of responses (N=555) exceeds the number of respondents (N=323), indicating that many applicants defined themselves using more than one category.
Exhibit 2. Applicant scientific focus, N=323

<table>
<thead>
<tr>
<th>Category</th>
<th>Number of answers</th>
</tr>
</thead>
<tbody>
<tr>
<td>Biological science</td>
<td>255</td>
</tr>
<tr>
<td>Behavioral science</td>
<td>39</td>
</tr>
<tr>
<td>Clinical science</td>
<td>87</td>
</tr>
<tr>
<td>Social science</td>
<td>18</td>
</tr>
<tr>
<td>Physical science</td>
<td>30</td>
</tr>
<tr>
<td>Chemical science</td>
<td>31</td>
</tr>
<tr>
<td>Computational science</td>
<td>34</td>
</tr>
<tr>
<td>Engineering</td>
<td>35</td>
</tr>
<tr>
<td>Mathematical science</td>
<td>16</td>
</tr>
<tr>
<td>No answer</td>
<td>10</td>
</tr>
</tbody>
</table>

*Many respondents selected more than one answer choice.*

The majority of the applicants intended to use T-R01 funding to explore new scientific ideas: 87% claimed that the proposed research was a significant departure from their research direction (Exhibit 3). Note that the RFA stated that proposed research should represent a completely new direction, which could have influenced respondents’ answers. In fact, it is somewhat surprising that as many as 13% of respondents admitted that their proposals were non-compliant with the RFA.

Exhibit 3: Similarity of proposed research to previous research directions, N=323

Was your T-R01 proposal a significant departure from your previous research directions?

The survey data suggested that the program attracted few applicants who had not been previously funded by NIH or at least had attempted to obtain funding from NIH. When asked to
describe their funding history, 299 or 93% reported having applied for an NIH award or grant as a Principal Investigator and 284 or 88% had applied to the T-R01 program last year (Exhibit 4).

**Exhibit 4: Past history applying for NIH, N=323**

<table>
<thead>
<tr>
<th>Prior NIH application?</th>
<th>No answer</th>
<th>No</th>
<th>Yes</th>
</tr>
</thead>
<tbody>
<tr>
<td>Percent respondents</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>100</td>
<td>7</td>
<td></td>
<td></td>
</tr>
<tr>
<td>95</td>
<td>93</td>
<td></td>
<td></td>
</tr>
<tr>
<td>90</td>
<td></td>
<td>11</td>
<td></td>
</tr>
<tr>
<td>85</td>
<td></td>
<td></td>
<td>88</td>
</tr>
<tr>
<td>80</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Furthermore, for many applicants NIH funding represented a significant source of their total research support: 62% of the respondents indicated that half or more of their funding came from NIH; for 10% all of their funding came from NIH (data not shown).

The majority of respondents (62%) learned about the T-R01 program directly from NIH, through the NIH guide, T-R01 program dissemination sources, or Program Officers (Exhibit 5). An additional 27% were notified about the program by their department or heard about it from colleagues or friends.

**Exhibit 5: Source of information about T-R01 program, N=323**

<table>
<thead>
<tr>
<th>Category</th>
<th>Number (%) of respondents</th>
</tr>
</thead>
<tbody>
<tr>
<td>NIH (guide, web site, staff, listserv)</td>
<td>200 (62%)</td>
</tr>
<tr>
<td>Departmental announcement</td>
<td>47 (15%)</td>
</tr>
<tr>
<td>Word of mouth</td>
<td>39 (12%)</td>
</tr>
<tr>
<td>Other (grantsnet, journal, institution)</td>
<td>34 (11%)</td>
</tr>
</tbody>
</table>

Almost half of the respondents reported that they were unlikely to receive funding support for their concept from other sources (Exhibit 6). The survey did not explore whether respondents had attempted to obtain funding for their idea elsewhere.
**Exhibit 6: Possibility of receiving funding from other sources, N = 323**

What is the likelihood that your T-R01 proposal might be supported by other funders?

- Very unlikely: 6%
- Somewhat unlikely: 22%
- Somewhat likely: 26%
- Very likely: 46%

**Application process**

To provide additional guidance to the applicants, program staff developed a document that contained answers to frequently asked questions (FAQs). When asked whether the RFA and the associated FAQs were helpful in describing the types of projects the program was seeking to fund, 365 (85%) responded in the affirmative (data not shown). The applicants found the information on the proposal review process the least informative (27% said that the instructions in this area were unhelpful, Exhibit 7). It is clear that NIH staff were available for guidance: of the 136 respondents who needed assistance, 113 (83%) were able to obtain it (data not shown).

**Exhibit 7: Adequacy of instructions (N=323)**

Were the T-R01 RFA and associated frequently asked questions helpful in describing the program goals, application requirements, and review process?

- Program goals: 96% Yes, 3% No, 2% No answer
- Application requirements: 95% Yes, 3% No, 2% No answer
- Review process: 71% Yes, 27% No, 2% No answer
Respondents who found the instructions not fully satisfactory were given an opportunity to elaborate on their views in a comments field. Five applicants noted that it was difficult to determine from the application what type of research NIH was looking to fund. For example:

\textit{Still not sure how innovative and unpredictable the research should be.}

\textit{It seems that this mechanism really only supports basic science. If that is the case, it should be made clear so that non-basic science researchers won’t waste their time preparing the application.}

Several applicants noted that it was unclear how NIH defined innovative, transformative, and paradigm breaking and suggested that some examples would be helpful. Two respondents also said that most grants that were funded last year were not, in their view, transformative. As one respondent put it:

\textit{I realized after the funding decisions were made that the guidelines put forth made little or no difference because only about 20\% of the proposals were truly transformative, judging by their summaries and by the bibliography of the PIs.}

This individual went on to say that the best funded proposals were a continuation of the PI’s work, which was a violation of the stated guidelines.

Finally, one applicant was unsure how much detail had to be included in the budget and another noted that the application directions were contradictory and that “reasonable, smart people could easily disagree what was or was not required.”

\textbf{Application format}

The applicants were asked to evaluate the importance of various proposal sections in communicating their concept’s novelty, innovation, and impact using a 1–6 scale, where 1 is unimportant and 6 extremely important. As Exhibit 8 shows, the Challenge, innovation, and impact statement was considered the most important by the majority of the applicants, followed by Rational and Approach. The Bibliography and Timeline were seen as least important.
Exhibit 8: Importance of each proposal section in communicating novelty, innovation, and impact (N=323)

<table>
<thead>
<tr>
<th>Section</th>
<th>1=Unimportant</th>
<th>2</th>
<th>3</th>
<th>4</th>
<th>5</th>
<th>6=Extremely important</th>
</tr>
</thead>
<tbody>
<tr>
<td>Project summary</td>
<td>6</td>
<td>7</td>
<td>14</td>
<td>24</td>
<td>24</td>
<td>24</td>
</tr>
<tr>
<td>Bibliography</td>
<td>17</td>
<td>22</td>
<td>23</td>
<td>22</td>
<td>10</td>
<td>5</td>
</tr>
<tr>
<td>Timeline</td>
<td>18</td>
<td>22</td>
<td>22</td>
<td>19</td>
<td>11</td>
<td>6</td>
</tr>
<tr>
<td>Appropriateness of T-R01 mechanism</td>
<td>3</td>
<td>8</td>
<td>7</td>
<td>15</td>
<td>25</td>
<td>41</td>
</tr>
<tr>
<td>Approach</td>
<td>14</td>
<td>10</td>
<td>21</td>
<td>30</td>
<td>33</td>
<td></td>
</tr>
<tr>
<td>Rationale</td>
<td>22</td>
<td>13</td>
<td>40</td>
<td>41</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Challenge, innovation, and impact statement</td>
<td>3</td>
<td>22</td>
<td>5</td>
<td>19</td>
<td>69</td>
<td></td>
</tr>
</tbody>
</table>

As noted above, transformative R-01 applications had to follow an abbreviated proposal format, with the research section not exceeding 8 pages (including figures) and bibliography not exceeding 1 page. The survey explored whether the applicants found this type of application adequate to communicate the novelty and transformative value of the proposed idea. The vast majority of the applicants said that both the format and the length were sufficient (Exhibit 9).

Exhibit 9: Adequacy of proposal format and length in communicating the novelty and transformative value of the proposed idea (N=323)
Of the 52 applicants (16%) who found the length of the research plan insufficient, 35% would have preferred an additional 2 pages, 56% an additional 4 pages, and 10% an additional 5 pages or more (data not shown).

**Suggestions for improvement**

In the survey, respondents were asked to share any ideas on how to make the review process more effective and efficient in identifying transformative research. The applicants submitted nearly 130 comments, organized by topic below.

**Reviewer choice.** Many respondents (N=35) wrote that the success of the review process depended entirely on the choice of reviewers. Good reviewers should be able to “think out of the box” and be open to innovative ideas. Interestingly, some applicants suggested that junior investigators were more likely to have these qualities as they have not yet become entrenched in the accepted ideas, while others felt that senior investigators would have the wisdom and the experience to identify transformative proposals.

*The biggest problem is that most researchers do not perform innovative and out-of-the-box research. Thus, where are reviewers going to come from?*

*Need to know how the reviewers have been trained out of their routine habits, their normal ways of reviewing grants.*

*The reviewer selection is the key. The reviewers that have done transformative research from different disciplines should be carefully selected and charged to review proposals.*

Several respondents expressed doubts that members of the distinguished panel (whose names were made public) had the necessary qualities to identify transformative research. Another concern was that the composition of the panel did not match the expertise required for some proposals. For example, two respondents noted that the review panel was composed almost exclusively of biomedical and clinical scientists, and therefore, research in behavioral and social sciences was unlikely to get funded. These comments revealed that some applicants were somewhat confused about the role of the generalist versus specialist reviewers. Included below are a few representative comments:

*... I do not believe the review panel listed at NIH Commons has the expertise to review my proposal.*

*The pool [of applicants] is too big and too diverse for any experts in broad knowledge to handle. It may be more appropriate to sub-divide.*

*Include some experts in the industry or pharmaceutical research institutes.*

Respondents made three recommendations related to the reviewers. One was that the applicants should have an option to suggest additional reviewers and to exclude the reviewers chosen by NIH, similarly to the traditional study section. The second suggestion was to include younger investigators on the panel and investigators with behavioral and social science
backgrounds. Finally, the applicants recommended choosing reviewers with a record of transformative work—for example, EUREKA or T-R01 grantees.

**Review process.** In addition to several comments that revealed participants’ confusion about the review process, 11 applicants commented explicitly that they did not understand the review process:

- I never got a clear idea about the number of review phases. My understanding was that the review process of this type of application is different from traditional R01, but I am still not sure HOW different?

- It is unclear what the review process is.

- It is unclear to me how the review proceeds for T-R01s. Is it a mail review in the first step?

The applicants noted that the review process should be more transparent. First, the names of all the reviewers—not just the generalist panel—should be made public. One individual even suggested making reviewers’ comments public, ostensibly the strategy adopted by the California Institute for Regenerative Medicine. Second, the applicants would have liked to have been notified about the status of their application during the review process—for example, how it was triaged by the panel in the first stage of the review. The applicants pointed out that this information would help them decide whether to apply for other funds to support the proposed idea:

- It would be great to have a triage process that gives a quick answer on the compatibility of the application for the ... program—if not appropriate, applicants could then spend more time pursuing other directions more aggressively.

Several respondents (N=6) advocated blind review. They argued that if the reviewers did not know the applicants’ names and institutional affiliations, they would be more likely to judge the proposals on merit and transformative potential:

- ... we need to start moving toward anonymous review.... It might level the playing field for applicants with less notoriety than Harvard. This would be especially important with the transformative approach—as it should be the innovative and new ideas that get funding.

- Go to a review process like the Gates Foundation in which scientific ideas are evaluated in the absence of the investigators’ identities.

A number of applicants (N=6) expressed disappointment with the duration of the review process. They noted that because the applications were very short, they should take no longer than three months to review.

*The submission date was January 22, 2010 [and] the review date not until June 2. For special projects with highly limited pools of money, it should not take six months to...*
assemble a review committee and complete the work. This set of projects should have been reviewed no later than March of this year.

Respondents also noted that since the review takes many months, the applicants should have an option to amend their proposals with more recent results.

Although I feel our preliminary data were strong at the time of the submission, an opportunity for supplementary data may have proved decisive.

Application format. A number of suggestions were made on the format of the proposal. Two applicants said that it should address the benefits of the research, in particularly to human health. Another respondent noted that the Challenge/innovation/impact and Rationale sections solicited similar information and should be revised to reduce overlap. Two applicants commented that the requirement for including a specific timeline was inconsistent with high risk research. One individual said that the application should contain only an innovation and impact statement; all other information was superfluous. Finally, one respondent commented that it was “a little unrealistic to believe that any research proposed for a transformative grant will not link back to an investigator’s other research.”

Findings: Reviewer Feedback

This section is a summary of reviewer answers to the survey. As mentioned in the Introduction, the T-R01 program used an “editorial” review model, whereby all applications were first triaged by a panel of generalist reviewers (Stage 1) and then reviewed by experts selected for their expertise in the proposal topic (Stage 2). Finally, the Stage 1 panel met to make final award decisions. This section is a summary of Stage 1/3 and Stage 2 reviewers’ responses. Note that the Stage 1/3 panel was composed of 12 individuals, of whom 5 responded to the survey. Because the sample size was small and the response rate relatively low, the information provided by this group may not be representative of the larger population of reviewers.

Demographic information

The survey did not collect demographic information on Stage 1/3 reviewers. Of Stage 2 reviewers, 79% were White, 13% Asian, and less than 1% American Indian and African American each (data not shown). The reviewer pool appears to be somewhat less diverse than the applicant pool (see the applicant demographics section above). Like the applicants, the reviewers were mostly male (74%, data not shown). Finally, as a group the reviewers were somewhat older than the applicants (Exhibit 10 and Exhibit 1).
Many of the reviewers in both groups had participated in this capacity in the first round of T-R01 competition (Exhibit 11). Two out of five Stage 1/3 reviewers thought that some of the applications they evaluated were resubmissions from the previous year (data not shown).
Workload

In the first stage of the review process, 545 applications were divided among 12 Stage 1/3 reviewers. In the survey, reviewers were asked whether this load was reasonable. Sixty percent of respondents reported that the number of applications was “just right” and the rest that it was too burdensome (Exhibit 12). The burden on the Stage 2 reviewers was significantly lighter, with 74% reviewing one application and 13% two applications (Exhibit 12). One Stage 2 reviewer reported evaluating 76 and another 26 applications, but this workload was significantly greater than average (data not shown).

Exhibit 12: Workload

Clarity of program documents

In the survey reviewers were asked whether the RFA and associated FAQs as well as reviewer instructions clearly communicated the intent of the T-R01 program. Virtually all reviewers in both groups responded in the affirmative (Exhibit 13).
Exhibit 13: Clarity of instructions provided to reviewers

A. RFA and FAQs
Did the RFA and other documents clearly communicate what type of research would receive support?

B. Stage 2 reviewers
Were the instructions provided to you clear?

Review process

Reviewers from each group were asked what fraction of the applicants proposed transformative research. Most reviewers reported that 10% of the proposals or less were in this category (Exhibit 14). This finding is interesting because the vast majority of reviewers felt that the RFA and other documents available to the applicants clearly communicated what type of research would receive support (Exhibit 13). Moreover, virtually all of the applicants said that the proposal instructions were adequate (Exhibit 7). These data underscore the challenge of conveying the program goals as well as the difficulty in being objective in evaluating the transformative potential and innovativeness of one’s own work.

Exhibit 14: Percentage of the applicants proposing transformative science
What percentage of the applications you reviewed proposed transforming science?
Survey data revealed that Stage 1/3 and Stage 2 reviewers focused on different sections in their evaluation of proposals, as intended by the program (Exhibit 15). Consistent with their role as generalists, 60% of Stage 1/3 reviewers found the Challenge/innovation/impact section most helpful for review (note the caveat of small sample size). The rest focused on the Biosketch, presumably to assess the applicant’s ability to implement proposed research. The role of Stage 2 reviewers was to evaluate the technical merit of the proposal. As various aspects of an application contribute to the assessment of technical merit, Stage 2 reviewers chose to focus on different parts of the proposal (Exhibit 15). It was somewhat surprising, however, that only 25% of Stage 2 reviewers found the Approach most helpful.

**Exhibit 15: Proposal section most useful to the reviewers**

*Which sections were most helpful for your review?*

<table>
<thead>
<tr>
<th>Section</th>
<th>Percent Respondents</th>
</tr>
</thead>
<tbody>
<tr>
<td>Project summary</td>
<td>60</td>
</tr>
<tr>
<td>Challenge, innovation, impact</td>
<td>40</td>
</tr>
<tr>
<td>Approach</td>
<td>22</td>
</tr>
<tr>
<td>Appropriateness of mechanism</td>
<td>17</td>
</tr>
<tr>
<td>Timeline</td>
<td>11</td>
</tr>
<tr>
<td>Bibliography</td>
<td>5</td>
</tr>
<tr>
<td>No answer</td>
<td>6</td>
</tr>
<tr>
<td>No answer</td>
<td>1</td>
</tr>
</tbody>
</table>

The survey examined whether and to what extent Stage 2 (expert) reviewers influenced the outcome of the review. All five Stage 1/3 reviewers reported that the input from the experts was “very helpful” (data not shown). The assessments of Stage 2 reviewers would be taken seriously only if Stage 1/3 reviewers considered their expertise relevant to the proposal. It emerged from the survey that Stage 1/3 reviewers found that Stage 2 reviewers “often” or “always” had the right expertise (Exhibit 16). Correspondingly, we found evidence that the input from the experts was considered: 4 of 5 panelists said that the Stage 2 reviewers “sometimes” dramatically changed their initial assessment of the proposals (Exhibit 16).
All Stage 1/3 reviewers also said that the face-to-face meeting (Stage 3 review) “sometimes” dramatically changed their views about an application (data not shown). Finally, all Stage 1/3 reviewers reported being completely satisfied with the final scores (Exhibit 17).
Suggestions for improvement

Like the applicants, the reviewers were asked to offer suggestions on how to change the RFA and the review process to increase the likelihood of receiving transformative proposals. Stage 1/3 and Stage 2 reviewers submitted 79 comments. The most frequent, made by 7 reviewers, was the view that the RFA should include a clear definition of transformative research and examples of projects that would and would not be funded by the program. (Note that this opinion was also strongly expressed by the applicants.) Reviewers also recommended that PIs be asked to give “honest reasons,” including in the summary statement, why their proposals are transformative. Another suggestion, made by 5 reviewers, was to introduce a pre-screening stage, such as a reviewed letter of intent or a 2-page pre-proposal. Reviewers noted that many proposals could have been easily eliminated at an early stage, saving time and effort for the applicants and the reviewers. Finally, a number of suggestions were made by a single respondent and these are summarized as bullet points below:

Application process and format
- Allow a longer biosketch (4 pages)
- Applicants should provide more detailed budget
- Make greater emphasis on experimental detail and lesser emphasis on rhetoric
- Allow late submissions for reviewers or change due dates (January is an active time for service on the study sections)
- Better advertise the program

Review process
- Let the applicants know that the first round is based almost exclusively on the abstract and challenge/innovation/impact statement
- Discourage submission of proposals that have been submitted in another format
- Advise the applicants that submitting “standard science” will only waste time
- The review outcomes (such as percent failure due to the lack of transformative potential) should be relayed to the research community
- Ask the applicants to explain what existing research/concept/technology will be “disrupted”
- Reduce the number of proposals that have to be considered in Stage 3
- Fund more proposals with smaller budgets as “feasibility studies” and make follow-up funding contingent on performance in Phase I
- Recruit senior investigators as reviewers
- Mail reviewers have no ability to calibrate their scores because they evaluate small numbers of proposals in isolation