Guiding Investments in Biomedical Research: Using Data To Develop Science Funding Programs and Policies*

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Data Based Decision Making and The Goals of Biomedical Funding Organizations

One important goal of organizations that provide funds for biomedical and behavioral research is to encourage and support research that leads to more effective health promotion, better disease prevention, and improved treatment of disease. They do this in order to provide a solid scientific base for preventive and curative interventions.

In order to ensure that a funding organization and its programs are effectively moving science forward toward this objective, it must continually assess and re-assess its goals, directions and progress. In general, the aim of program assessment in this context is to determine whether the organization's programs and its policies are having the impact on science that they have planned for or are expecting.

While there are many ways to carry out assessments and many endpoints that can be used, for several reasons to be discussed in this paper, it is important that the assessments capture data that can link organizational decisions to the subject matter of science. Further, in order to ensure that the organization is examining the effects of its funding efforts on science per se, the primary data used in the assessment and subsequent decision making process should be progress in science itself. When used properly and often enough, ideally the outcome of an assessment should be able to reflect whether the organization is maximizing its impact on advancing science as well as to determine whether what has been done in the past has met the specific goals and objectives of the organization, and, last but not least, provide information that can chart the organization's future scientific program priorities and directions.

The use of scientific progress as an indicator of an organization's impact is the central point of this paper. However, before expanding on that point, let us first briefly examine an assessment approach that can be used in charting an organization's impact. The approach is not new and can be used by any type of organization or program. What is new is the fact that the rate of scientific discovery over the past decade or so has increased exponentially. The half-life of an idea in science continues to get shorter and today it seems to be on the order of weeks or months not years or decades as in the past. Organizations that are not able to keep up with this pace fall behind and can become irrelevant so not only do organizations need to continually assess and re-assess goals and objectives, they need to do so it in real time in order to stay current.

There are several discrete and interlinked components in program assessment. They are outlined in Figure 1, which also illustrates one possible way in which they can be used,

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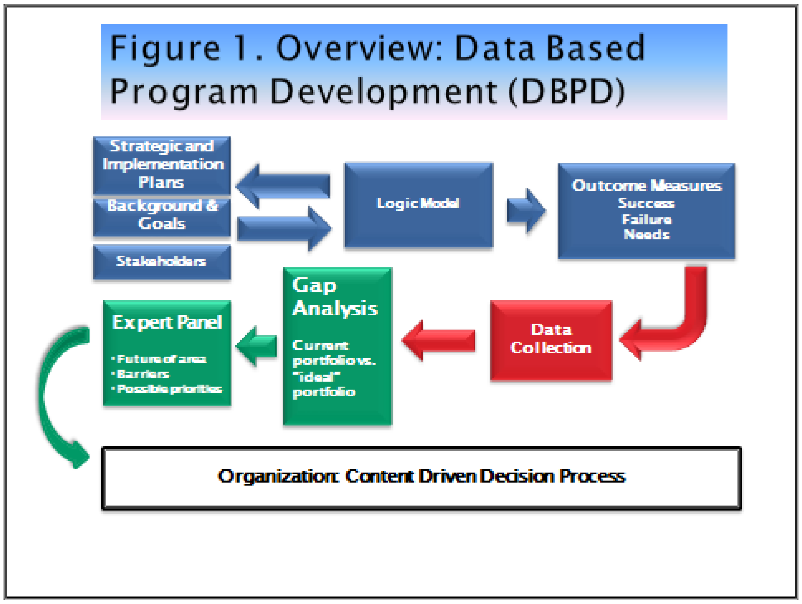


The components include:

- a ***strategic plan*** that identifies organizational values, mission, priorities and objectives;
- an ***implementation plan*** listing the timelines, benchmarks, mechanisms of implementation, and the sequence of events related to the elements of the strategic plan;
- ***a logic model***, based on information gained from all stakeholders and which identifies inputs or available resources that can be used along with expected outcomes from the organization's activities. The logic model also helps the organization define those outcomes that will be used as measures of success. In this sense it plays a key role in carrying out what some have termed; and
- a ***gap analysis***, an assessment of progress in reaching organizational goals and specifically the benchmarks and timelines of the implementation plan. This is accomplished by assessing the current position of the organization in relation to where it expected or wanted to be at a given point in time. In the process of conducting a gap analysis the organization also addresses specific questions about the current state-of-the-science along with pathways to scientific advancement in terms of what is needed to move science ahead along with identifying barriers to and opportunities for progress. Very often, the gap analysis relies, at least in part, on input from an ***expert panel*** or thought leaders who, in addition to reviewing the organization's current grant portfolio and future developmental plans, also share their ideas about the organization's proposed changes, the barriers and opportunities for meeting organizational goals, and how all of this fits within their view of the area of science and it's near- and long-term future.

Taken together this is what I call ***data based program development (DBPD)***. That is to say, development of the organization's goals in supporting and/or stimulating scientific progress is primarily guided by using data as opposed to whim or anything else.

Demographics vs. Progress In Science As The Primary Endpoint

My premise is that the data that should be used in DBPD ***is science itself*** because it is the endpoint that most closely aligns to the overarching goals of funding organizations…to support research that maximizes the impact of organizational activities and programs on progress in science. This may seem like a very strange statement, particularly to individuals who have the responsibility for developing scientific programs that fund research through competitive grants or contracts. After all, doesn't science already drive the process of program development through peer review of applications submitted for funding? To some extent it does. However, that is at the micro level. What about at the macro or organization level…what guides decision making there?

Certainly organizations don't want to be in the position of supporting or encouraging research that's not going to advance knowledge. So, if funding organizations don't use progress in science itself to assess current programs and/or plan for future programs, how do they determine if they are supporting the most impactful research? In my experience and in talking with colleagues at science agencies from around the world it appears that most of the outcome measures used are what I call 'demographic information', that is information that that answers the following kinds of questions for any individual portfolio of grants or even for collections of grant portfolios:

- How much is being spent on a particular funding program or a particular area of science?
- How many grants are in the program portfolio of grants?
- What is the geographical distribution of the grants?
- What is the mix of granting mechanisms (e.g., basic vs. translational vs. clinical research; investigator initiated vs. solicited research; single project grants vs. large center multi-center grants) within a portfolio?
- How many inventions or patents have resulted from the research?
- What is the citation rate of grantees? Is the organization funding the most highly cited scientists?
- How many grantees have received recognition from other scientists in and out of their field?
- Are the grantees publishing in the best journals?
- How many applications were received either through specific solicitations or as initiated by an investigator or both?
- How many of the principal investigators are former trainees supported by the organization?

What can be puzzling is determining how one gets from these quantitative measures to outcomes based on information contained in the results and discussion sections of journal articles and reports published by scientists themselves. Or is it even possible to do that at all?

While we would like to believe that there is a direct connection between these 'demographic' or quantitative measures and progress in science itself, I would argue that the demographics are surrogates for a direct analysis of what is actually happening in science. They don't tell us much, if anything, directly about the specific contributions that grants supported by an organization have on science itself. For example, with perhaps the exception of an analysis of patents or inventions, answers to the questions listed above cannot tell us whether results reported from a particular grant removed an important and long existing block to progress in an area of research. I am not suggesting that we should completely ignore the demographic outcome measures. They are very important in charting the progress of the funding organization in realizing many of their goals and objectives…it's just that they don't provide information about advances in science itself.

But that's not to say that there aren't data that reflect changes in science nor is it to say that there aren't ways to collect and synthesize those data. In the past progress in science has been documented by engaging the services of a program analyst or group of analysts and asking them to categorize the titles, abstracts, progress reports or key words of grants or grant applications and track them over time. In some cases, directors of grant portfolios have done this informally.

Program analysts are typically highly experienced and/or trained specifically for the analysis, and they carry out the analysis by hand very diligently and systematically. Even so, from time to time, from document to document, or from person to person the algorithm they use in classification and categorization can change enough to introduce a source of variability in the analysis. As a result the reliability and perhaps even the validity of the final results can be compromised. This doesn't happen because the people doing the work are sloppy, lazy or malicious. It happens because they are human…and humans, even those who are well trained, highly experienced and very careful, can and do exhibit variability in behavior over time or between similar tasks. Or as a colleague of mine once said "shift happens." Moreover, analyzing science by hand is a long, tedious, and expensive task. So our tendency has been to do this kind of detailed analysis infrequently or once in a career…clearly not in 'real time' as seems to be what is needed in this day of fast-paced discovery.

Analysis of Text and Scientific Fingerprinting

Fortunately, the technology now exists that allows us to analyze the content of science in a valid, reliable and timely way that overcomes many of the problems that crop up when it is done by hand. More than that, because this approach is computer-based, and therefore fast and reliable, its use allows us to ask and answer questions that are, at best, difficult and, at worst, impossible to address when we use human power to collect the data. The approach I'm referring to involves the formal textual analysis of science ***concepts and knowledge*** contained within documents such as strategic and implementation plans, grant applications, progress reports, and the scientific literature. We refer to the output of the textual analysis of a document as a '***scientific fingerprint***' or simply a '***fingerprint***'.

Without going into the details of the underlying processing (mainly because it is well beyond my area of expertise and understanding), a fingerprint is a precise abstract representation of text that allows us to look into the text (content) rather than only looking at the meta data (demographics). Figure 2 illustrates the development of a fingerprint from document through development of an ontology to the actual fingerprint itself. In this figure the fingerprint is the weighted collection of terms contained in the boxes that comprise the ontology.

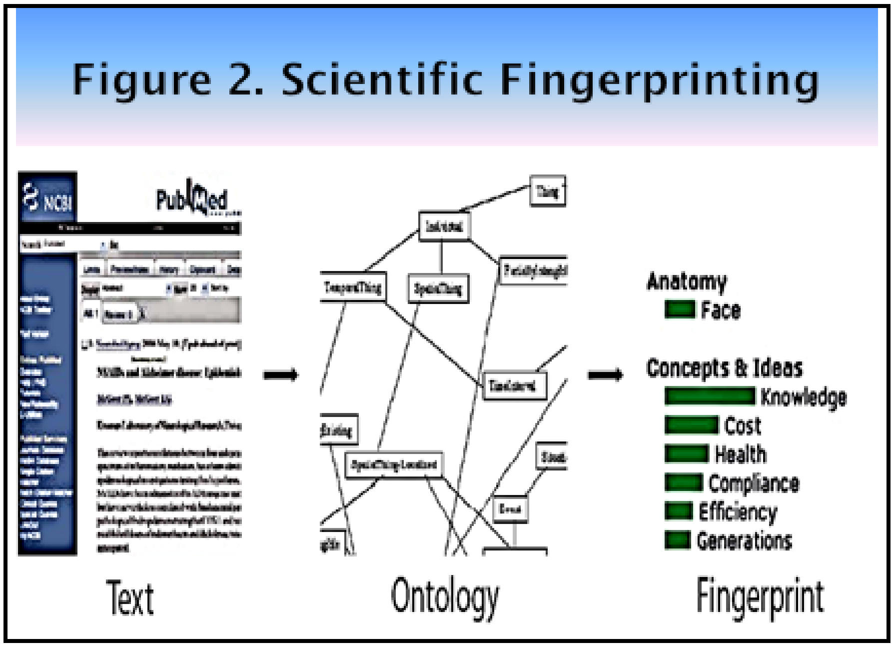


Figure 2. Scientific Fingerprinting

Because fingerprinting is concept driven and not key-word driven and because it uses as its base an ontology (i.e., A is an example of B), it is not necessary to have a term appear in a document in order for it to be part of a fingerprint. For example, it is possible for a document to contain all of the diagnostic characteristics of a disease but not the name of the disease in order for the disease name to appear in the fingerprint. The only requirement is that the diagnostic characteristics be identified as examples of the named disease somewhere in the scientific literature comprising the database that is searched.

Fingerprinting uses as its base for comparison and analysis the entirety of the Elsevier Scopus database consisting of 45.5 million records. The information used to develop a fingerprint can be captured relatively easily, inexpensively and quickly. Because it is computer-based the textual analysis of a grant portfolio, for example, is also much faster than when done by hand, thus allowing an organization to continually assess and reassess its scientific grant portfolio much more often than is historically done. Further, and also because fingerprinting is computer-based, it is not subject to the variability that enters into the process when humans do the textual analysis. The same document will result in the same fingerprint every time it is analyzed no matter who enters it into the computer…'shift doesn't happen' but change in the fingerprint can occur when something has changed in science and that change is documentable and can be extremely useful to the organization in analyzing their current portfolio and charting their future.

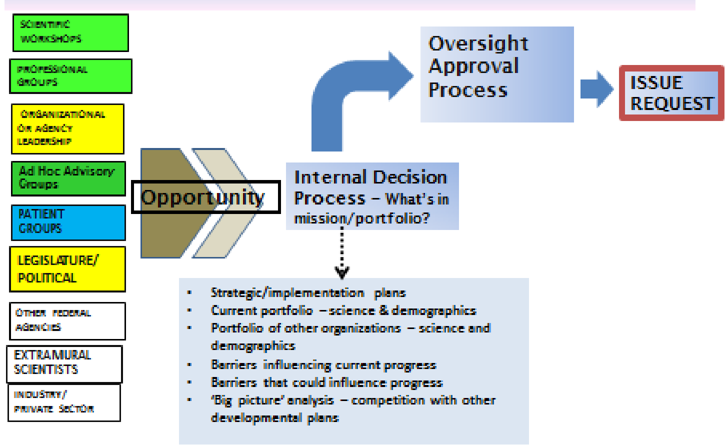
Textual analysis can be used for any document as long as there is an adequate database to provide the basis for the ontology. And it can be used at any stage of evaluation and program development. In short, it is possible to carry out continual ongoing real time assessments as opposed to the 'once-every-five years' approach to updating strategic or implementation plans. Further, the concepts comprising a scientific fingerprint of textual content can be adjusted to fit the views of experts in the field, thus they are not adopted blindly or without validation by experts. And finally, fingerprints can be modified over time both by experts and by the content of the body of scientific literature itself that changes over time to reflect advances in science. In fact, those changes can be documented and used in an analysis of progress attributable to the organizations funding efforts.

What I am suggesting is a process that is similar in many ways to employing the help of program analysts mentioned earlier in that it turns contextual data (i.e., information about what is actually happening in science) into information that can provide an assessment of the impact of the organization's activities on progress in science. Together with the demographic information about the organization's programs, information can be amassed that can be the basis for a very rich assessment. The difference is in the speed and repeatability of the data collection effort.

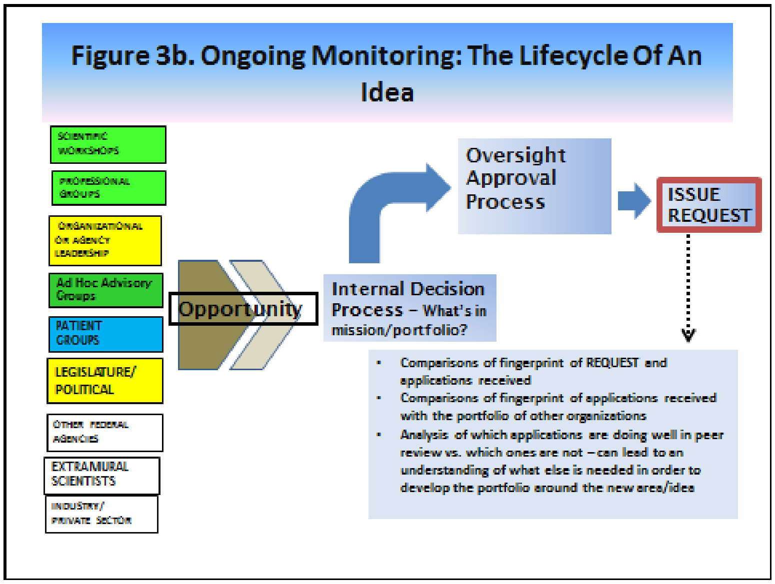
Let us look at an example of how fingerprinting can be used in the ***development and monitoring of a scientific program*** by documenting programmatic changes that are introduced through the generation of new ideas or new advances in science and then tracking progress in implementing the new ideas. The example is illustrated in the following two figures, Figure 3a and 3b, which show, in the column of boxes on the left, illustrate the multitude of sources for ideas about what an organization should be focusing on in its funding plans. These inputs are processed by the organization and eventually can result in issuance of a request for applications that address specific scientific needs or opportunities.

Figure 3a focuses on the ability to use fingerprinting to sort out many suggestions for new areas by comparison of fingerprints of the ideas with fingerprints of organizational planning documents as well as of grant portfolios of both the parent and sister organizations. These comparisons of fingerprints can provide the data that will facilitate internal decision making in the process of sorting through the many ideas or opportunities facing the organizations. In short, it helps the organization address the question 'which, if any of these, opportunities do we address?'

Figure 3a. Ongoing Monitoring: The Lifecycle Of An Idea



By taking into account factors such as the relevance of an opportunity or group of opportunities to its mission, planning documents, current portfolio of its grants as well as the portfolios other organizations with similar goals and determining which of many possible ideas to develop immediately or in the future, the organization is able to clarify its pathway to the future in the selection of specific opportunities to include in requests for applications. In addition, it is at this point that the organization can assess its current and past grant portfolios as well as those of sister organizations in order to identify overlaps and decide whether or not to proceed in areas already supported by them and/or by other organizations. This kind of analysis would seem to be particularly important during times of fiscal restraint and/or retrenchment. Once one or more idea is selected it is possible to move to issuing a Request for Applications (REQUEST) or to engage in any other means the organization uses to encourage scientists to submit applications for funding.



As illustrated in Figure 3b, fingerprinting can also be used to monitor progress toward the implementation of the newly selected opportunity. By monitoring in real time the scientific content of grant applications that are received in response to the REQUEST the organization is able to tell whether or not they are meeting their original scientific programmatic goals in issuing the request. Moreover, by comparing the scientific content of successful applications with those that aren't successful it is possible to isolate related factors that need to be addressed before progress in science can occur. For example, it may be that an idea is ahead of technology currently available to address it. Another possibility is that unsuccessful applications result from a lack of specific scientific expertise or experimental designs. Knowing this in a timely manner can result in developing or expanding other programs in the organization's portfolio. And finally, it is possible to compare the content of applications received with the fingerprints of applications received by sister organization or even with other grant portfolios within the organization to determine if there is significant overlap.

Tables 4a and 4b provide examples of other possible uses of fingerprinting in monitoring the development or ongoing management of grant portfolios. One of the most creative uses of fingerprinting is illustrated by an analysis of contributions to progress in science by leaders in research on Alzheimer's and Parkinson's diseases conducted by Aaron Sorenson. References

Table 4a. Other Monitoring Possibilities Using Fingerprinting

- *Is this a new idea?* – terms used in articles or research applications that don't appear in thesaurus or ontology may represent a new and innovative idea or approach to a problem
- Comparison of fingerprints of applications to determine *scientific characteristics of successful vs unsuccessful ones* – addresses the question are we too far ahead of the field or simply getting poorly constructed applications?
- Understanding the factors that influence funding success (e.g., training, expertise, publications, participation in a multi-disciplinary group) – development of new training programs; basis for advising potential applicants

for two published articles about the analysis by Sorensen are provided in Table 4b.

Table 4b. Other Monitoring Possibilities Using Fingerprinting

- Comparison of fingerprints of successful applications that get published (or published in best journals) vs those that aren't
- Contributions to the scientific field:
  - Aaron A. Sorensen. Alzheimer's Disease Research: Scientific Productivity and Impact of the Top 100 Investigators in the Field, Journal of Alzheimer's Disease 16 (2009) 451–465 451
  - Aaron A. Sorensen and David Weedonb. Productivity and Impact of the Top 100 Cited Parkinson's Disease Investigators since 1985. Journal of Parkinson's Disease 1 (2011) 3–13.

Fingerprinting can be used for purposes other than the assessment of program development, success or impact. For example, fingerprinting is at the core of a tool referred to as Reviewer Finder, a key component in identifying scientific expertise linked to specific areas of science. By fingerprinting scientific articles associated with an individual, this tool can be used to identify individuals with the expertise required for peer review of applications as well for assessment of grant portfolios and future goals of the organization's plans. Reviewer Finder can also be used to form interdisciplinary research teams within a research organization as well as globally. Another use of fingerprinting involves the rapid and reliable classification and categorization of documents such as those used in a gap analysis as well as in ongoing monitoring of the organization's grant portfolio(s) referred to earlier. This application can also be used to track publications and contributions to science similar to the analysis conducted by Aaron Sorensen referred to earlier.

If Fingerprinting Is What Is Needed, Why Isn't It Already In Use?

While the technology behind the development of fingerprints is well developed, the use of fingerprint technology for scientific program assessment, monitoring and development is in its infancy. It is possible to tailor the fingerprinting tool to organization-specific needs but there is still a need for developing and validating data obtained from fingerprinting in a way that would allow translation of the qualitative aspects of fingerprinting into quantitative measures. For example, quantification is needed to represent the amount or extent of change in a fingerprint resulting from change in science. This quantification would need to reflect both changes in the weight of concepts as well as the introduction of new concepts as the result of changes in science. On the one hand, changes in the weight of a concept can be viewed as a measure of the extent of change while the addition of a new concept to a fingerprint can be viewed as an measure of impact. This level of detail is important to an organization in gauging the full influence of their funding on science.

At the same time it is important to note that one limiting factor here would be the ability to attribute a change in science to a specific grant. This limitation is not unique to fingerprinting or any other approach to program assessment. Rather it is the result of the fact that research labs are organized around addressing specific scientific questions and not around the sources of grant funding. So, while a lab may report an important finding in a given research article or group of articles and may attribute the finding(s) to a specific grant, the reality of the situation is that, because the lab may be supported by several grants, together they may all have contributed to the advancement in science. Even here, however, fingerprinting might even be useful in untangling the overlap among grants.

The main point, however, is that fingerprinting can allow organizational decision making to be based on the state-of-science and provide the organization with direct information about its role in and contributions to a specific area of science. In addition to using progress in science itself to play a key role in the assessment and development of scientific programs, it can help align organizational goals with the current state-of-the-science. As such, when coupled with demographic data charting the organization’s performance, the use of text analysis can provide a fuller picture of the state-of-science, the current role of the organization in moving science forward, and in the possible role that the organization can play in future scientific development.