

Early Program Outcome Assessment: The Common Fund Closeout Guide

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Presentation Abstract:

Measuring the outcomes of short-term research programs can be challenging. The National Institutes of Health (NIH) Common Fund supports trans-NIH research programs that last 5 to 10 years and are goal-driven. These programs are managed mostly by biomedical researchers with backgrounds in fields like chemistry, biochemistry, microbiology, molecular biology, and biophysics. Often these scientists have no background in program evaluation. To ensure that we capture outcomes, a guide was developed to help program teams with this. Information from two broad categories—State of the Science and Program Management-- is collected, analyzed, and reported in the Program Closeout Report. Components of the report are shared with internal and external audiences with an interest in the program outcomes. This presentation will discuss the purpose of this guide, assessment questions, methods and metrics, challenges and solutions to capturing needed information, and utilization of findings.

Overview

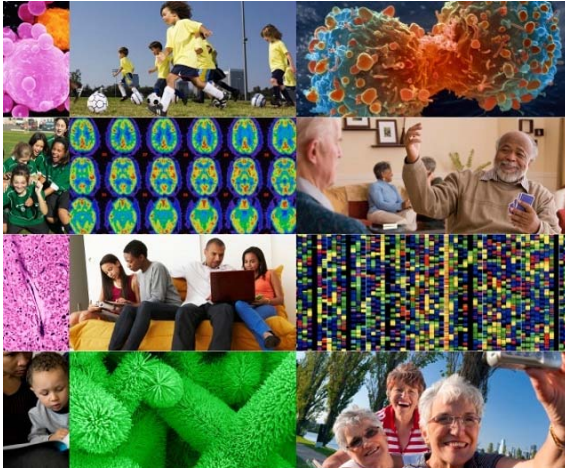
- NIH Common Fund
- Closeout Report Guidelines
- Methods
- Use of Findings



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Today we are going to share the experiences of the NIH Common Fund in measuring short term outcomes of biomedical research programs and a tool we have developed to capture this

National Institutes of Health (NIH)



“Science in pursuit of **fundamental knowledge** about the nature and behavior of living systems ...

and the **application of that knowledge** to extend healthy life and reduce the burdens of illness and disability.”

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The National Institutes of Health (NIH) is a Federal Agency within the U.S. Department of Health and Human Services, and the premier funder of biomedical research in the United States. The agency is subdivided into 27 Institutes and Centers, each with a unique mission to advance an area of biomedical research and human health. Some focus on a particular disease (e.g., National Cancer Institute). Some focus on organs (e.g., National Heart, Lung and Blood Institute; National Eye Institute). Others have a broad focus that crosses many diseases/organ systems (e.g., National Institute of General Medical Sciences, National Institute on Aging, National Human Genome Research Institute).

Most or all ICs develop forward-looking strategic plans, to define priorities and set research agendas.

What is the NIH Common Fund?



- Supports cross-cutting, trans-NIH research programs
- Provides a strategic and nimble approach to addressing key roadblocks in biomedical research
- “Venture capital” space for high-risk, innovative endeavors with potential for extraordinary impact
- Short-term (5-10 year), goal-driven programs
- Programs focus on developing specific deliverables to catalyze research

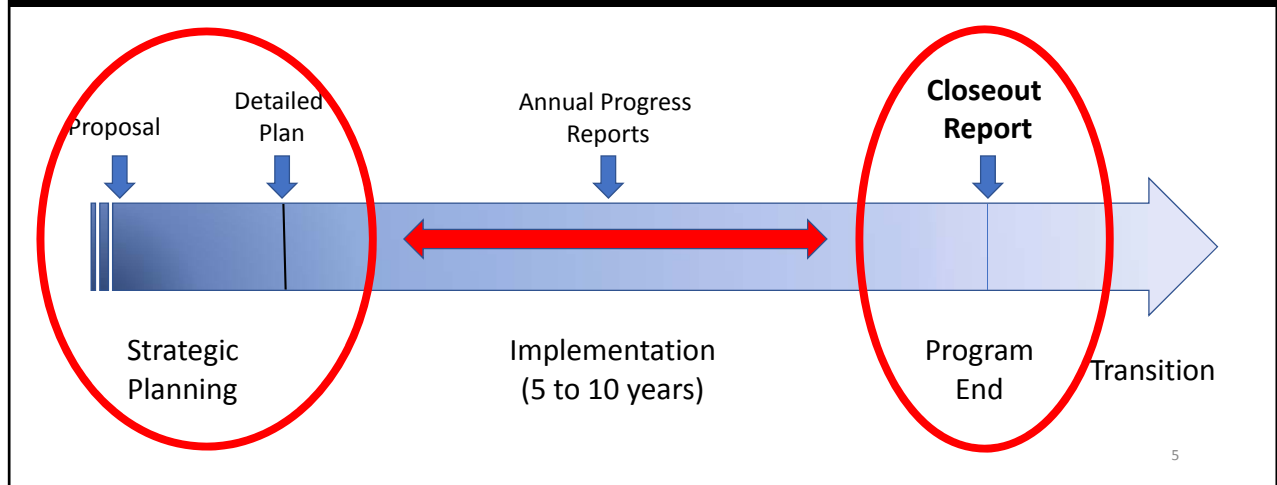
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The NIH Common Fund is a unique funding entity within the NIH Office of the Director. Started in 2004, its purpose is to remove key roadblocks to biomedical research and to capitalize on emerging scientific opportunities that no single NIH Institute or Center could tackle on their own, but which are high priority for the NIH as a whole. Common Fund scientific investments are used to reveal fundamentally new biomedical paradigms, develop new and innovative technologies, methods, and tools, that change the way scientists approach their work, and to generate comprehensive data sets or other resources that catalyze investigator-initiated research and enable discovery. The work supported by the Common Fund can be inherently risky, but this risk is embraced due to the potential for transformative impact in advancing science and, ultimately, improving human health.

The Common Fund supports a series of short term, exceptionally high impact, trans-NIH programs. Each program has a 10-year maximum life span in which to achieve specific goals. A set of milestones assessing progress toward program goals is required. All Common Fund Programs are required to meet the following five criteria:

- Transformative: Exceptionally high & broadly applicable impact
- Catalytic: Achieve a set of high impact goals within a defined period of time
- Synergistic: Value-added to the NIH Institutes and Centers
- Cross-Cutting: Address complex issues requiring management by trans-NIH teams
- Unique: Provide new solutions to specific challenges the National Institutes of Health (NIH)

Planning for Closeout Report Starts at the Beginning of a Program to Enable Adequate Usable Data and Information



Each Common Fund program has a 10-year maximum life span in which to achieve specific goals. A set of milestones assessing progress toward program goals during the planning stage. These are updated, as needed, throughout the life of the program. At the end of the program, a Closeout Report addresses the outcomes of the program to-date.

So that adequate information has been collected for the closeout, planning and collection of data and information begins with the proposal and detailed plan and continues through the life of the program.

Purpose of Closeout Report



- Documents:
 - ✓ Goals
 - ✓ Achievements
 - ✓ Outcomes
 - ✓ Challenges
- Provides lessons learned
- Demonstrates accountability
- Becomes the record of the program
- Helps plan for future

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Throughout the life of the program, the program's impact on the research community, on the treatment or prevention of disease, and on patients is actively monitored. As a program ends, the Closeout Report documents goals, achievements, outcomes, and challenges that the program faced. The Closeout Report not only serves to document outcomes but can also provide lessons learned for future programs. It is generally developed by the program staff in consultation with a group of experts who are asked to provide input.

The impact of a given science program may not be felt for a number of years after the program ends. The Program Closeout Report can help NIH plan for future tracking of the program's success (e.g., use of program-generated resources or data, expansion of science area) after the program has ended.

In planning for the end of a program, a key issue is the transition of datasets, infrastructure, or other items that require long term support. NIH considers if the program (or specific components) will need to continue after Common Fund support ends. To do this, NIH staff identify the most valuable resources (e.g., knowledge, data, tools, technologies, cell lines, administrative procedures, special emphasis panels) created by the program and work to ensure these resources will be made available beyond the period of Common Fund support. In addition, entities such as the NIH Institutes and Centers, other funding agencies, or private entities such as drug companies, foundations, etc. that are most likely to continue support of the resources generated by the program are identified.

General Questions considered in the Closeout Report



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Each of Common Fund program is different. So, we use a template with open-ended questions to guide collection and analysis of data for the Closeout Report.

General questions considered in the Closeout Report include:

- What impact has the program had on the research community, treatment or prevention of disease, on patients, and/or on the way that NIH supports research?
- What information needs to be, or has been, gathered to document this impact?
- Will resources, data sets, or other deliverables from the program require ongoing funds?
- Will the outcomes or deliverables from the program need to be tracked after the program ends to assess impact? How might that best be accomplished?

Two Categories of Questions related to Closeout

State of the Science

Evolution and attainment of program goals

Products of research

Significant contributions to the field of science

Utilization of knowledge generated, research products, training

Management

Effective strategies used to ensure progress

Adequacy of type and level of support to awardees to attain goals

Communication and coordination effectiveness

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Data Sources for Closeout Reports

Publications, Patents, Copyrights
Administrative records, Grant data
End-users of new tools and technologies
Meeting summaries
External program consultants/Experts
NIH staff and Principal Investigators



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Information for the Closeout Report is collected from baseline through the program end. A variety of sources are used such as publications, patents, copyrights, administrative records, grant data, end-users of new tools and technologies, meeting summaries, external program consultants/experts, NIH staff and principal investigators.

There is no one-size-fits-all approach to measuring effectiveness. Different Common Fund programs need different approaches at different points in their lifecycle. Often, conducting a very simple evaluative activity (survey, panel, etc.) may be all that is needed.

Analytic Methods

Quantitative and qualitative analysis

- Surveys/questionnaires
- Request for information (RFI)
- Social media discussions
- Interview, focus group
- External advisors' input
- Workshops and meetings

Comparisons over time

- Portfolio analysis
- Landscape analysis
- Literature review
- Bibliometric analysis



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Analysis of collected information involves quantitative and qualitative approaches with comparison over time to identify any changes in the field of science.

Example Common Fund Program: Molecular Libraries and Imaging Program 2004-2014

Overarching Principle

Broad public sharing of
chemical biology data



<https://commonfund.nih.gov/molecularlibraries/index>

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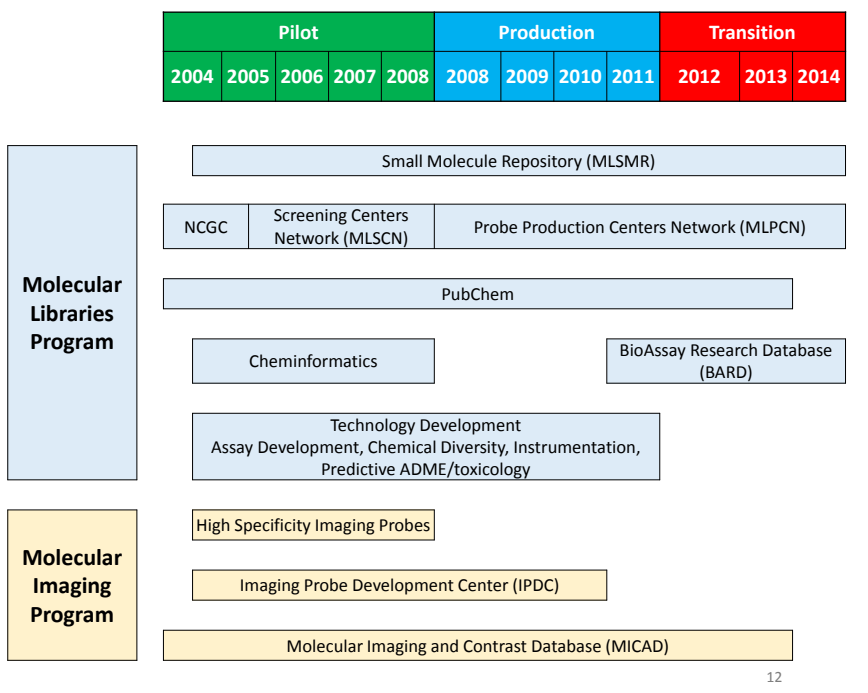
We are going to use the Molecular Libraries and Imaging Program as an example of how we answer questions from the Closeout Report. To understand this example, we are providing a brief overview of the program.

Goal of Molecular Libraries and Imaging Program: To empower the research community to use small molecule compounds in their research, whether as tools to perturb genes and pathways, or as starting points to the development of new therapeutics for human disease.

The NIH Common Fund Molecular Libraries and Imaging Program began in 2004 and ended in 2014. This program:

- Offered biomedical researchers access to the large-scale screening capacity to identify small molecules
- These molecules were then optimized as chemical probes, that could be used to study the functions of genes, cells, and biochemical pathways
- This led to new ways to explore the functions of genes and signaling pathways in health and disease

Molecular Libraries and Imaging Initiatives



As shown in this figure, the Molecular Libraries and Imaging Program involved multiple initiatives and projects over 10 years. These fell under 2 major components – the Molecular Libraries Program and the Molecular Imaging Program.

Overall Program Budget: \$895.8M over 10 years



Example Closeout Report Questions and Answers

Question: Were there obstacles to achieving goals and milestones?

Answer:

- Little willingness to take risks and apply untested and unproven technology in research proposals
- Underestimating the time and manpower requirements to complete a project

Question: What strategies were used to address these obstacles?

Answer:

- Help from Steering Committee, NIH Project Team and External Scientific Panel
- Adjusted goals and milestones to assure high quality and specific probes, as best-in-class
- Peer reviewed quarterly probe reports
- Incentive awards provided for new and novel proposals as supplements to the ongoing projects

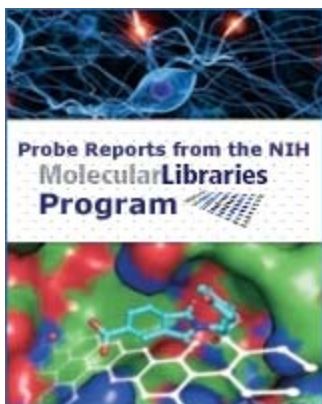
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To give you a sense of some of the questions and answers in the Closeout Report, this slide shows two related questions and answers about the program.

As a reminder, the main goal of Molecular Libraries and Imaging Program was to empower the research community to use small molecular compounds (probes) in research, either as tools that would interfere with genes and pathways to study their function, or as starting points to the development of new therapeutics for human disease.



Example Closeout Report Findings



Probe Reports from the NIH Molecular Libraries Program [Internet].
Bethesda (MD): National Center for Biotechnology Information (US);
2010. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK47352/>

Question: What are the most significant contributions of this program to the field?

Answer:

- High quality probes from the program in use around the world
- Many probes commercially available
- Some probes being used in animals and man as drug candidates



Example Closeout Report Program Deliverables

Question:

What were the products of the research program?

Answer:

NCATS Small Molecule Resource's public access to compounds of the MLI
NIH Clinical Collection
NCGC Assay Guidance Manual and HTS Guidance Criteria
Probe Reports and Probe Report abstracts in PubChem
Probes - available from ML Centers and commercial vendors
PubChem
BioAssay Research Database (BARD)
Molecular Imaging and Contrast Database (MICAD)
Imaging Probe Development Center (IPDC)

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The Molecular Libraries and Imaging Program produced many research resources that have been used by the scientific community as shown in this slide. Some products such as PubChem, a database of chemical molecules and their activities against biological assays, continue to be used and are now managed by other NIH Institutes.

Question:

What were the products of the research program (e.g., tools, technologies, databases, repositories, etc.)?

Example Closeout Report Science Management

Molecular
Libraries
and Imaging



Question:

What management strategies were most effective in ensuring progress of the program?

Answer:

- Set annual goals and milestones for each initiative
- Had monthly teleconferences with PI and NIH Project Scientists
- Set high, measurable standards for program output

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Example Closeout Report Overall Outcome

Molecular
Libraries
and Imaging



Today, compounds resulting from probes developed by the Molecular Libraries and Imaging Program are in clinical trials with the possibility of going all the way to market.

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The program mission was to provide high-throughput screening and resources to the scientific community to develop probes to study the function of genes, cells and biochemical pathways.

Today, compounds resulting from probes developed by the Molecular Libraries and Imaging Program are in clinical trials with the possibility of going all the way to market.



Use of Closeout
Report findings

Accountability
Planning Future

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Closeout Report findings are used:

- To demonstrate to stakeholders such as NIH leadership, HHS, Congress, and the White House that the investment and scientific progress made by the program has been an effective use of resources
- To plan future science investments
- To use lessons learned to better manage science programs.

Challenges Evaluating Early Outcomes of Science Programs



Developing meaningful standards for success in early outcomes is difficult

Biomedical research training not preparation for program evaluation

Science program managers often more interested in the science than the program

Once a program ends, science managers lose interest in reporting

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Common Fund programs are limited to 10 years total and are meant to achieve a specific goal within that timeframe. However, the ultimate intended impact of the program may be felt after the program has ended. Developing meaningful standards to evaluate early outcomes remains a challenge.

While biomedical research training makes scientists content experts in the research areas they are supporting, it does not prepare them to carry out evaluative activities to assess the outcomes of their programs of science. Scientists generally have strong analytical skills and are comfortable with the idea of systematic inquiry. However there are significant cultural differences in the way biomedical researchers view this and the AEA Guiding Principle of systematic inquiry

Recommendations for Evaluating Early Outcomes of Science Programs

Engage stakeholders

Validate evaluation findings

Use a variety of metrics

Use more than one tool

Data need to be looked at in context

Compare like with like

- Similar research areas
- Similar journals (discipline)
- Stage of academic career



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When evaluating early outcomes of science programs, we have learned the following:

- Engage stakeholders through-out the program
- Validate evaluation findings with staff and investigators
- Data need to be looked at in context
- Use a variety of metrics and other qualitative information where appropriate
- Don't use just one tool--coverage varies in content, depth, discipline
- Because at times we are talking about Apples and Oranges, that is different things, it is important to compare like with like. The metrics used are normalized by grouping research areas and discipline in similar categories.

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For more information about the NIH Common Fund is available at
<https://commonfund.nih.gov>

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