



Health, Education, and
Human Services Division

B-280562

September 11, 1998

The Honorable Joe L. Barton
Chairman, Subcommittee on
Oversight and Investigations
Committee on Commerce
House of Representatives

Subject: Results Act: Biomedical Research in HHS' Fiscal Year 1999
Performance Plan

Dear Mr. Chairman:

In September 1997, under the Government Performance and Results Act (the Results Act) of 1993, the Department of Health and Human Services (HHS) submitted its first strategic plan. This plan laid out the department's mission, long-range goals, and objectives as well as strategies for accomplishing them. In February 1998, HHS submitted its first annual Results Act performance plan for the fiscal year 1999 budget. The performance plan should provide annual goals with measurable target levels for assessing progress toward the achievement of the long-range strategic goals and objectives. The Results Act and the Office of Management and Budget's (OMB) guidance to federal agencies call for outcome-oriented goals in both strategic and performance plans.¹ This is an attempt to shift the focus of federal agencies away from the traditional concerns of staffing and activity levels toward a single overriding issue—achieving results.

One of HHS' six strategic goals was to "strengthen the nation's health sciences research enterprise and enhance its productivity." However, we and others

¹OMB Circular No. A-11 defines outcome as "the intended result, effect, or consequence that will occur from carrying out a program or activity." Under OMB guidance for the Results Act, when there are no good outcome-oriented goals, output goals can be used instead. OMB Circular No. A-11 defines output as "the level of activity or effort that will be produced or provided over a period of time or a specified date."

have reported that assessing research programs in terms of their outcome goals is extremely difficult. Research, the attempt to obtain knowledge about the unknown, by its very nature is unpredictable. Research activities may or may not yield outcomes. There can also be a considerable time lag between research activities and the outcomes of those activities. This presents a difficult challenge for assessing research outcomes on an annual basis, which the Results Act promotes by focusing on establishing and measuring annual performance goals. Even more problematic is the difficulty in tracing a specific research activity or program to research outcomes. Nonetheless, government-funded agencies engaged in research functions and activities are not exempt from accountability for their public expenditures.

A number of efforts have been undertaken in the past couple of years to help address the challenges posed by the Results Act for research programs. In July 1996, the National Science and Technology Council reported on the various quantitative and qualitative indicators for assessing research, concluding that appropriate methods would be agency- and program-specific and that some period of experimentation would be required.² In January 1998, the Committee on Science, Engineering, and Public Policy of the National Academy of Sciences, National Academy of Engineering, and Institute of Medicine began a study on identifying and analyzing the most effective approaches for assessing the results of research, determining how federal agencies may better incorporate research activities into their strategic and performance plans, and evaluating the effect of implementing the Results Act. The committee expects to issue a report later this year.

In the context of the challenges associated with assessing the outcomes or results of research, you asked us to examine how HHS' performance plan addressed medical research. Specifically, you asked that we determine whether the performance plan (1) provided objective and measurable performance goals by which the outcomes of medical research programs could be assessed, (2) described strategies for achieving the specified research goals, (3) identified factors that could affect the ability to achieve the research goals and provided an indication of how these factors would be addressed, and (4) indicated how different research agencies within HHS would coordinate their efforts to achieve the research goals.

²Assessing Fundamental Science, Committee on Fundamental Science, National Science and Technology Council, July 1996.

To develop our information, we examined performance plan information on the two HHS agencies whose major function is to conduct and support medical research—the National Institutes of Health (NIH) and the Agency for Health Care Policy and Research (AHCPR). We also examined plan information for the Centers for Disease Control and Prevention (CDC), where research is an important part of the agency's activities, although not its major function. Although other HHS agencies are engaged in research activities, we focused on these three agencies because they were targeted for increases in medical research funding under the president's fiscal year 1999 budget proposal.

In summary, the agencies did not always identify measurable outcomes that would allow an assessment of their research accomplishments. In some cases, their objective was to increase understanding or create new knowledge without a specific application in mind, such as NIH's goal to increase the understanding of normal and abnormal biological functions, a goal that does not easily lend itself to measurement. In some instances, when the objective was directed toward obtaining knowledge to meet specific needs, the agencies were able to specify measurable outcome-oriented goals. For example, to obtain information on health care utilization and cost, AHCPR said it would increase the number of families it would interview by 5,600. However, even where the research was directed toward a specific need, the goals were not always specified in a measurable form. For example, although one of NIH's goals was to improve and develop new advanced instrumentations and computers, NIH did not provide information on how improvements or developments would be measured.

Generally the agencies did not explicitly identify their strategies for accomplishing their specific research goals. However, the performance indicators included in the plan as a means of assessing progress toward achieving their goals provided some insight as to what those strategies might be. For example, although NIH did not explicitly discuss its strategies for developing an AIDS vaccine, the agency's performance indicators—the size of its research portfolio, the number of interactions between academic investigators and industry, and the completion of ongoing clinical trials and initiation of additional trials—suggested that the strategies the agency would use to develop an AIDS vaccine involved increasing specific research activities in this area.

Although not required under the Results Act, the usefulness of the performance plan would be enhanced by a discussion of major factors outside the control of the agency that could affect the agency's ability to achieve its goals. Two important factors that the agencies have limited control over are the inherently unpredictable nature of research and research conducted externally by grantees

(extramural research) rather than by agency personnel. None of the agencies specifically discussed in the plan how these two factors might affect their ability to accomplish their goals or how they would account for them. In describing the goals, performance indicators, and strategies for achieving the goals, the plan gave the impression that research would achieve the desired outcomes. There was no acknowledgment of the possibility of not accomplishing the goals because of the risks associated with research and, as a result, there was no discussion of how the agencies intend to mitigate the risks. When research is conducted externally by research grantees, the agency has less control over activities throughout the course of the research that can affect the outcomes, such as changing the direction of the research and shifting personnel and resources. Although the agency can influence the research outcomes through the priorities it sets for its research agenda, the grants it awards, and the monitoring of research activities, there was no discussion in the plan linking such activities to the attainment of its goals.

All three agencies addressed coordination with other entities to accomplish their research goals, although the way in which the discussion was presented in the plan differed. Both AHCPR and CDC described their coordination efforts in relation to their various research goals. For example, CDC in describing its goal to conduct research to reduce worker illness, injury, and death discussed its consultation with a broad array of individuals and organizations in the occupational health and safety community. NIH, in contrast, had a discrete goal to increase collaboration and cooperation within the agency and with other agencies and private organizations. Rather than describing an overall strategy, NIH provided several examples of collaborative efforts.

As the process of strategic planning, annual goal setting, and performance reporting proceeds under the Results Act, we expect HHS' performance plan to become more specific about what the department intends to accomplish and how the various HHS agencies will achieve their intended research goals. In particular, we would look for the research agencies, as is the case with other HHS agencies, to be able to better specify measurable goals and performance indicators and to discuss factors outside the agencies' control that might affect the accomplishment of their research goals.

BACKGROUND

NIH is the principal federal biomedical research agency. It comprises 22 institutes and centers, each created by the Congress with separate appropriations and charged with a specific mission. The institutes and centers focus on a given disease, like cancer or mental illness; a particular organ, like

the heart or eye; or a stage of development, like childhood or old age. Approximately 80 percent of NIH's appropriated funds goes toward extramural research grants and about 11 percent toward its in-house research program staffed by NIH's own physicians and scientists. NIH also provides training support through research training grants, individual fellowships, and career development awards.

AHCPR's research efforts focus on determining what works best in clinical practice, improving the cost-effective use of health care resources, helping consumers make more informed choices, and measuring and improving the quality of care. More than 80 percent of AHCPR's research budget goes toward supporting extramural research activities. AHCPR also provides training support through its grants and fellowships.

Research is one way CDC accomplishes its mission—the promotion of health and quality of life by preventing and controlling diseases, injury, and disability. In addition to research, CDC monitors health, detects and investigates health problems, develops and advocates health policies, implements prevention strategies, promotes healthy behavior, fosters safe and healthy environments, and provides public health leadership and training. CDC accomplishes its goals by working with partners in local, state, and foreign health organizations. Approximately 36 percent of CDC's research is conducted extramurally.

NIH's fiscal year 1998 research budget is about \$13 billion. The president's fiscal year 1999 budget for NIH proposed an 8-percent increase in medical research, which would raise the agency's budget to approximately \$14 billion. AHCPR's fiscal year 1999 proposed budget provided for a 12-percent increase for research from \$90 million in fiscal year 1998 to \$101 million. Although research is but one of CDC's functions, its research budget is nearly four times that of AHCPR.³ CDC's fiscal year 1999 proposed budget would result in a 2-percent increase in its research funding from \$358 million in fiscal year 1998 to \$364 million. CDC's fiscal year 1999 budget request included \$25 million for a new prevention research program.⁴

HHS' fiscal year 1999 annual performance plan consisted of a short HHS-wide overview along with separate sections for major components of HHS—including

³In fiscal year 1998, research constitutes about 14 percent of CDC's total budget. CDC's fiscal year 1999 proposed agencywide budget is \$2.6 billion.

⁴The budget figures pertain to the budget authority for research activities, excluding funds for buildings and facilities.

NIH, AHCPR, and CDC. In our June 1998 report on HHS' performance plan, we assessed the department's overall plan rather than each of the components individually. As was the case with many other federal agencies, HHS' performance plan contained considerable valuable information. However, we concluded that many parts of the plan could better fulfill the purpose of the Results Act by (1) consistently setting measurable performance goals, (2) providing information about how HHS agencies would coordinate with one another and other organizations to achieve their respective goals, (3) identifying resources they need to accomplish their goals, and (4) discussing how they intended to address problems with their performance data.⁵

AGENCIES DID NOT ALWAYS IDENTIFY MEASURABLE OUTCOMES

According to the Results Act and OMB guidance, outcome-oriented goals need to be specified in measurable terms so that they can be assessed. To enable an assessment of progress, measurable goals should include both baseline data and a target level. Also, performance indicators should be provided to assess progress toward achieving agency goals. However, the agencies did not always specify their goals in a measurable form, particularly when the research objective was directed toward the creation of new knowledge without a specific application in mind.

For research with no specific application in mind, not only is it difficult to determine a knowledge base but it is also difficult to determine whether the research would result in a measurable increase of that base. For example, from our review of the plan, it was not clear how NIH would determine whether it had achieved its goal of increasing the understanding of normal and abnormal biological functions. For this goal to be measurable, one would need to measure the current level of understanding of biological functions as well as any increases of that understanding.

When the research objectives were directed toward obtaining knowledge to meet specific needs, in some cases, the agencies were able to provide measurable goals. For example, to indicate progress toward the development of genomic information, NIH indicated that it will complete "sequencing of the human genome by 2005 by initially reaching a production rate of 100 million basepairs in 1999 and growing to a production rate of over 300 million

⁵The Results Act: Observations on the Department of Health and Human Services' Fiscal Year 1999 Annual Performance Plan (GAO/HEHS-98-180R, June 17, 1998).

basepairs a year by 2003."⁶ Likewise, to obtain information on the use of and expenditures for health care services, AHCPR would increase the number of families it would interview by 5,600 from its previous 1998 sample of 9,000 families. One of CDC's goals was to prevent avoidable illnesses and deaths caused by exposure to toxic substances in the environment. CDC said that it would determine through research how to obtain accurate measures of human exposure to toxic substances in the environment. Consequently, it set a performance goal of increasing the number of toxic substances that could be measured at the agency's environmental health laboratory by 25 percent from the 1997 baseline of 200.

However, in other cases, even where the research was directed toward a specific need, neither the goals nor the performance indicators were specified in a way that would allow for an assessment of progress. For example, NIH established a goal of improving and developing new advanced instrumentation and computers. However, it gave no indication of what would constitute improvements or new developments and how those would be measured.⁷

Similarly, AHCPR stated that because little is known about effective methods for getting clinicians to alter their practices on the basis of clinical evidence obtained through research, it established a performance goal aimed at improving "understanding of how to ensure research affects clinical practice as appropriate." However, the agency did not specify how it would measure "improved understanding" so that it could be assessed.

AGENCIES' STRATEGIES ARE SUGGESTED BY PERFORMANCE INDICATORS

NIH, AHCPR, and CDC provided few explicit discussions of strategies for accomplishing their research goals. However, while the agencies do not lay out explicit strategies, the performance indicators often give an indication of what those strategies might be. For example, AHCPR did not discuss strategies for creating knowledge to improve health outcomes. The performance indicators the agency provided were the completion of specific research projects. Based

⁶Basepairs make up the structure of genes that are the basic units of heredity material. It is the sequencing of these basepairs that contains hereditary information.

⁷One of NIH's two sample indicators for this goal was the development of a novel imaging system. The development of such a system is measurable if NIH also provides some means for determining whether the system is novel.

on the indicators, it appears that the agency's strategy for accomplishing its goal—creating new knowledge—was to support research on health outcomes.

In other cases, strategies combined research with other activities. For example, NIH's performance indicators for developing an AIDS vaccine were an increase in the research portfolio, interactions between academic investigators and industry, and the completion of ongoing clinical trials and initiation of additional trials. In another case, one of AHCPR's goals was to obtain information on the use of and expenditures for health care services. AHCPR's performance indicators for this goal were the number of interviews conducted with nationally representative samples of families, medical providers, and insurance providers. AHCPR's apparent strategy for obtaining information, then, was to conduct interviews with three representative samples.

THE PLAN DOES NOT DISCUSS TWO FACTORS AFFECTING THE ACHIEVEMENT OF RESEARCH GOALS

The utility of the performance plan would be improved with a discussion of factors that can affect the agencies' ability to accomplish their goals, although such discussion is not required under the Results Act. A discussion of factors that an agency has limited control over should also include strategies the agency would undertake to mitigate these factors.

Two important key factors that the agencies have limited control over are the unpredictable nature of research and the inability to directly control the large portion of the research that is conducted extramurally through research grants. None of the agencies discussed how they would account for risks associated with the unpredictable nature of research in their discussion of outcome-oriented goals, performance indicators, or strategies for accomplishing their goals. The plan gave the impression that because an agency conducted or supported a particular type of research, certain outcomes would be obtained upon completion of that research. For example, developing an AIDS vaccine by 2007 was an NIH performance goal. NIH's performance indicators for this goal were increases in the research portfolio for AIDS-related research, expanded interactions between academic investigators and industry, and the completion of ongoing clinical trials and initiation of additional clinical trials in this area. However, increases in research activities in this area would not necessarily lead to progress toward the development of a vaccine. Because of the intrinsic nature of research, we would expect some acknowledgment that increasing AIDS research activities might not yield the desired outcomes and a discussion of contingencies that the agency may have for dealing with unexpected outcomes. In the case of developing an AIDS vaccine, for example, NIH might

assess whether increases in the research portfolio, interaction between academia and industry, and clinical trials are contributing to sufficient progress or whether there ought to be changes in the strategies used to accomplish this goal. Additionally, monitoring progress in this area can allow for midcourse adjustment of either the goals or strategies used.

The second factor that affects an agency's ability to achieve its research goals is whether the agency conducts the research itself or funds research conducted by an external principal investigator. An agency has greater control over the research activities it conducts internally than it has over extramural research activities it funds and monitors. For example, with intramural research the agency has greater flexibility in revising research goals, changing the direction of the research, and shifting personnel and resources in response to information acquired during the course of the research. Considering that about 80 to 90 percent of NIH's and AHCPR's research is done through extramural grants, these agencies might have less control in making needed adjustments toward achieving their research goals for a major portion of their research portfolio. About 36 percent of CDC's research is conducted extramurally. However, according to HHS, most CDC-funded external research is conducted through cooperative agreements and contracts under which the agency has more control because it is able to fund research incrementally and, if necessary, terminate or reduce funding.

While an agency has no direct control over extramural research activities, it can influence research outcomes through activities it does control—setting priorities for the research agenda, awarding grants, and monitoring research. The plan would benefit from a discussion linking such activities that the agencies do control to the attainment of their goals.⁸ The plan, however, provided little discussion of how this factor might affect the accomplishment of the agencies' research goals and how the agencies would account for it.

AGENCIES DISCUSSED COORDINATION IN VARYING WAYS

Although all three agencies discussed efforts to coordinate both within the agency and among other entities, the way in which the agencies discussed their

⁸In specifying its goals and performance indicators, NIH distinguished between research outcomes and administrative processes that included the establishment of research priorities, grants administration and peer review, dissemination of research results, technology transfer, management functions, and collaboration and cooperation with other organizations.

coordination activities differed. AHCPR and CDC described their coordination and collaboration activities relative to their various research goals. For example, AHCPR stated that to achieve its goal of improving health care quality, it would coordinate with the Veterans' Administration and the Department of Defense through the Interagency Quality Improvement Council. In relation to CDC's goal of reducing illnesses, injuries, and deaths in high-priority areas and high-risk sectors, it discussed its efforts in developing a research agenda with the assistance of other organizations from the occupational safety and health community.

In contrast to AHCPR and CDC, NIH had increase collaboration and cooperation in the pursuit of science as a discrete goal. Rather than describing an overall strategy, NIH described specific examples for promoting opportunities to collaborate and cooperate with other agencies, private organizations, and community groups as well as within the agency. For example, NIH said that it would expand existing clinical research collaborations with the Department of Defense, Veterans' Administration, and Indian Health Service. It said that it would also participate in the Advisory Committee on Blood Safety and Availability and establish a partnership with the Arthritis Foundation to support a national registry.

AGENCY COMMENTS AND OUR RESPONSE

We provided copies of this letter to HHS for review. With the three exceptions noted below, HHS generally agreed with the information we reported. HHS also provided technical comments, which we incorporated where appropriate.

First, HHS officials believed that it was important that we recognize that predicting research outcomes and linking accomplishments to program funding on an annual basis is particularly challenging, even for seemingly more tangible objectives like developing new advanced instrumentation and computers. We agree, and we modified the letter accordingly.

Second, while agreeing that assessing the results of extramural research is difficult, HHS did not agree that it represented a separate challenge and argued that numerous non agency participants—elsewhere in the federal government as well as in the private sector—all share accountability for program results. Recognizing that many parties share responsibilities, we nevertheless believe that there is a meaningful distinction in the government's role and activities undertaken in research programs that are carried out directly by federal researchers and those that are carried out by grantees in the private sector.

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For this reason, we continue to believe that the HHS plan should explicitly recognize this distinction, in addition to the others discussed in this plan.

Third, the department believes that we should discuss in the letter the importance of qualitative goals and elaborate on current efforts under way to develop means for assessing research programs. We agree and have expanded our description of that effort.

As arranged with your office, unless you publicly announce its contents earlier, we plan no further distribution of this report until 30 days from the date of this letter. We will then send copies to the Secretary of Health and Human Services and others who are interested. We will make copies available to others upon request.

Please contact me at (202) 512-7119 if you or your staff have any questions. Other major contributors to this report were James O. McClyde, Assistant Director, and Bertha Dong, Evaluator-in-Charge.

Sincerely yours,



Bernice Steinhardt
Director, Health Services Quality
and Public Health Issues

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