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Briefing Report to the Chairman, Subcommittee on Health and the Environment, Committee on Energy and Commerce, House of Representatives

October 1988

CANCER TREATMENT

National Cancer Institute's Role in Encouraging the Use of Breakthroughs

GAO/PEMD-89-4BR

GAO

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Program Evaluation and
Methodology Division

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The Honorable Henry A. Waxman
Chairman, Subcommittee on Health and
the Environment
Committee on Energy and Commerce
House of Representatives

Dear Mr. Chairman:

In your June 2, 1987, letter, you asked us to examine several issues related to the application of "breakthroughs" in cancer therapies. (See appendix I.) This briefing report is the second in a series of reports prepared in response to your request. It expands on a briefing to the subcommittee staff in March 1988. In an earlier report we sent to you, entitled Cancer Treatment 1975-85: The Use of Breakthrough Treatments for Seven Types of Cancer (GAO/PEMD-88-12BR, Jan. 1988), we discussed the extent to which cancer patients actually receive state-of-the-art therapies. In a forthcoming report, we will discuss the impact these therapies have had on the patient population.

The role played by the National Cancer Institute (NCI) in promoting the utilization in general clinical practice of treatments proven to be effective in experimental situations is the subject of this report. We have focused on the activities that NCI undertakes to increase physicians' use of state-of-the-art therapies. Our primary sources of information for this report were NCI documents and interviews with appropriate NCI staff. Because our focus was on NCI activities aimed at physicians, we did not examine programs intended to educate cancer patients or the general public, and we did not include activities by other groups. One element that was critical for us in addressing the issues was a list of which treatments should be considered state of the art. The list of breakthroughs in treatment was provided by the NCI director's office.

The Diffusion Process

We discuss NCI's efforts in terms of a general process by which treatments move from research to the patient. This process has four general steps: identification, diffusion, adoption, and implementation.

In the identification step, NCI redefines an "experimental" treatment as a "recommended" treatment. However, the move from experimental to recommended treatment is not always clear-cut. Differences of opinion about scientific evidence frequently occur before a consensus is reached

on whether a therapy is ready for widespread use, so it is particularly important to have a set of criteria for defining a recommended treatment. We found that the agency has an official consensus process established to routinely review scientific evidence to determine whether some change should be made in what NCI has called the "state of the art." However, the agency does not always coordinate its statements with the official position. As a consequence, physicians looking to NCI for advice on state-of-the-art treatment may get many responses, or conflicting responses, depending on what office or data base they query.

In the second step of the process—diffusion—a therapy has been recommended and the task is to convince physicians and patients of its utility. We found that NCI both supports and undertakes many programs to disseminate information about cancer therapies. However, our results suggest that few physicians are being reached through any of these programs. (Appendix II outlines NCI's dissemination activities.)

While NCI is required by law to disseminate information on cancer treatments, it is not required to actively promote the use of particular therapies. We found little agreement at NCI about what the agency's role in diffusion should be. Some NCI staff and directors expressed the belief that diffusion of treatments into practice is not, and should not be, the agency's responsibility.

The third step of the process—adoption—recognizes that while diffusion is important, it is not sufficient to move breakthrough therapies from clinical trials to the patient. A treatment will not reach the patient if the physician, informed of the breakthrough, does not adopt it. We discussed evidence of a problem with the adoption of new cancer therapies in the report we sent to you in January. As we noted, a considerable number of patients afflicted with the seven cancers that we examined did not receive what NCI considers state-of-the-art treatments. For example, 20 percent of those with Hodgkin's disease, 25 percent of those with small-cell lung cancer, and 37 percent of breast cancer patients did not receive what NCI had told us was the most appropriate treatment.

The fourth step of the research-to-patient process is implementation. That is, even when the identification, diffusion, and adoption steps are well executed, the questions still remain as to whether the therapy is implemented properly and whether it achieves the anticipated effects on patient survival rates. Our forthcoming report, which examines the impact that breakthroughs in treatments have had on the survival rate of the cancer patient population, will discuss this issue.

Results in Brief

Based on our examination, we believe that problems exist at many different points on the continuum along which breakthrough treatments must pass to reach cancer patients. There is a problem in coordinating the agency's positions on recommended treatments. Additionally, efforts at disseminating new treatments to physicians have met with only limited success. Finally, in many cases recommended treatments have not been adopted.

Matter for Congressional Consideration

We have included in section 5 a matter for congressional consideration. If the Congress wants to see a greater governmental role in the diffusion of optimal cancer treatments, then it may want to consider directing NCI, or some other office created for this purpose, to undertake concerted efforts to clearly identify and promote the use of cancer therapies that are sufficiently proven to warrant being classified as recommended treatments.

Agency Comments

We received written comments on this report from the Department of Health and Human Services after the 30 calendar days specified by law; therefore, they have not been reproduced in the report. However, we obtained informal comments from the agency, which we have incorporated, where appropriate.

As we agreed with your office, unless you publicly announce the contents of this report earlier, we plan no further distribution of it until 30 days from the date of the report. At that time, we will send copies to the Secretary of Health and Human Services and other interested parties and will make copies available to others upon request. If you have any questions or would like additional information, please call me at (202) 275-1854.

This report was prepared under the direction of Michael J. Wargo, Associate Director. Other major contributors are listed in appendix III.

Sincerely yours,

Eleanor Chelimsky
Director

Contents

Abbreviations

GAO	General Accounting Office
NCI	National Cancer Institute
NIH	National Institutes of Health
PDQ	Physician Data Query

Introduction

Background

The national cancer program has involved a large-scale effort by the federal government to fund research related to the diagnosis, prevention, and treatment of cancer. In order for this research to have a positive impact on the survival rate of cancer patients, the findings must move from the research setting into the treatment setting. How treatments proven to be effective in experimental situations make their way into general clinical practice and the role played by the National Cancer Institute (NCI) in that process are the subjects of this report.

We prepared this report in response to a request from the Subcommittee on Health and the Environment of the House Committee on Energy and Commerce. (See appendix I.) The Chairman asked us to determine whether it is true that cancer patient survival rates would improve if there were more widespread and better utilization of "breakthroughs" in cancer therapies. Our research in response to this request has focused on three issues:

- the extent to which cancer patients actually receive state-of-the-art therapies,
- the impact these therapies have had on the survival rate of the cancer patient population, and
- the activities that NCI undertakes to increase utilization of state-of-the-art therapy.

The first issue was discussed in a report sent to the subcommittee in January of this year.[1] The second issue will be covered in a forthcoming GAO report. We focus here on the third issue.

Scope and Methodology

In consultation with the subcommittee, we focused our efforts on NCI activities aimed at physicians. We did not examine programs intended to educate cancer patients or the general public, and we did not examine efforts by other agencies, institutions, or professional associations.

To learn what activities NCI undertakes to increase the utilization of treatment advances, we collected relevant NCI documents and interviewed appropriate agency staff. Our efforts centered on the NCI offices responsible for research into treatment advances, as well as those concerned with information communication. This gave us insights into the agency's general procedures. We also reviewed the literature on the

[1] Cancer Treatment 1975-85: The Use of Breakthrough Treatments for Seven Types of Cancer, GAO/PEMD-88-12BR (Washington, D.C.: Jan. 1988).

information diffusion process, particularly for medical breakthroughs, to understand what steps are involved in diffusion and what types of activities are usually successful.

Because our focus was on the activities NCI undertakes to promote physicians' use of optimal therapies, we sought what we believe was critical to addressing the issues: a list of those treatments considered as state of the art. This list provided specific situations in which there were breakthrough therapies to be promoted. It was developed by the NCI director's office and met the following criteria:

- The treatments were proven to increase patient survival in a large randomized clinical trial.
- The results of those trials became available by 1982.[2]
- The treatments were relevant to an identifiable group of cancer patients.

The list of breakthroughs that NCI provided as proven extenders of patient survival included treatments for 10 different cancers: adjuvant chemotherapy for breast cancer, adjuvant chemotherapy for colon cancer, chemotherapy for Hodgkin's disease, chemotherapy for non-Hodgkin's lymphoma, chemotherapy for osteosarcoma, hormonal therapy for prostate cancer, adjuvant radiation therapy for rectum cancer, chemotherapy for small-cell lung cancer, chemotherapy for soft-tissue sarcoma, and chemotherapy for testicular cancer. Only seven of these cancers were analyzed, however, because prior to our January report NCI withdrew one (hormonal therapy for prostate cancer), stating that it did not meet our criteria, and we eliminated two (chemotherapy for osteosarcoma and soft-tissue sarcoma) because of insufficient numbers of patients for reliable analyses.

A listing of the NCI activities we examined is provided in appendix II. These include publications, data bases, and other programs for the dissemination of treatment information. We did not verify the data on utilization of the NCI-supported data bases.

We received written comments on this report from the Department of Health and Human Services after the 30 calendar days specified by law; therefore, they have not been reproduced in the report. However, we

[2]This particular time interval was necessary for us to complete our analyses of the use of state-of-the-art therapies discussed in our January 1988 report. It also was valuable for looking at diffusion, because it allowed sufficient time after proof of efficacy in a clinical trial for NCI to carry out activities to increase utilization.

obtained informal comments from the agency, which we have incorporated, where appropriate, into the report. Our work was performed in accordance with generally accepted government auditing standards.

Getting Treatments to Patients: A Four-Step Process

The process by which treatments move from research to the patient can be envisioned as having four general steps: identification, diffusion, adoption, and implementation. We examined the role played by the National Cancer Institute in this process.

In the identification step, a determination is made as to whether an experimental therapy is ready for use in general practice. In the diffusion step, activities are undertaken to disseminate information about the therapy and to convince physicians to use it. In the adoption and implementation steps, the focus moves from the agency to the physicians, who must make a determination about whether to use the therapy and must learn to use it appropriately.

Identification and diffusion are the primary steps relevant to the subcommittee's question regarding what activities NCI undertakes to promote utilization. These steps thus serve as the focus of the report and, in turn, are discussed in sections 2 and 3. The adoption and implementation steps are relevant to the subcommittee's questions regarding the extent to which cancer patients receive the therapies and their impact on patient survival. These issues are briefly discussed in section 4; more comprehensive coverage of these issues is provided in our other reports to the subcommittee. Our summary and a matter for congressional consideration are presented in section 5.

Identification

Mechanisms for Recommending Treatments

In the identification step, it is necessary for NCI to redefine an "experimental" treatment as a "recommended" treatment. To do this, there must be some yardstick by which such a redefinition occurs. Having systematic criteria for reviewing the evidence and for making a determination of when sufficient evidence exists to actually recommend a treatment are essential for deciding that a new therapy represents an advance in the state of the art. Without such criteria the move from one step to another is not clear-cut, and many differences of opinion can occur about what evidence is needed to progress from the experimental to the recommended treatment stage.

PDQ Data Base

The official mechanism that the agency uses for assessing scientific evidence on treatment advances is its Physician Data Query (PDQ) system. PDQ is an on-line data base that includes a "textbook" of oncology that tells physicians what the recommended state-of-the-art therapy is for any form of cancer. It provides the latest information on prognoses, staging and cellular classifications, and descriptions of comparable treatment options for each type and stage of cancer.

The PDQ statements are developed by an editorial board of cancer specialists, many from outside NCI, who further consult with experts in oncology around the country on the best available therapies for all major forms of cancer. The statements are reviewed and updated monthly. We were told that the editorial board for PDQ operates independently of NCI control, but that the PDQ statements represent official NCI positions. However, other individuals with responsibilities for publications such as the institute's journal do not typically coordinate with PDQ.

We consulted PDQ to determine what information NCI was publicizing on the seven treatment advances we examined. We learned that in many instances there are multiple treatments listed equally under "state-of-the-art treatment options" for patients with a particular type of the cancer for which the breakthrough had occurred.[1] For four of the seven treatments, the PDQ options were consistent with the list of treatment breakthroughs we had received from NCI's Office of the Director, reflecting the position that these treatments were judged to be the most effective available. However, in three instances (for colon cancer, rectum cancer, and testicular cancer) other treatments were also listed as appropriate. This means that the treatments NCI had identified to us as

[1]The term "particular type" refers to specific characteristics relevant for treatment, such as stage, size of tumor, location of tumor, and lymph node involvement.

breakthroughs were not the only treatments being recommended in the PDQ system, but that other treatments were judged to be equally effective. The breakthroughs in these instances, then, could be considered as alternatives, not advances, to other existing treatments.

Consensus Development Conferences

The National Institutes of Health (NIH), of which NCI is a part, hosts consensus development conferences on topics about which there is some scientific dispute. These conferences provide another mechanism for achieving consensus about the state of the art. The conferences cover a range of health issues, thus NCI is only able to sponsor one or two such conferences per year. Since these consensus development conferences were begun in 1977, NCI has sponsored or cosponsored 17 of them. Of the seven breakthroughs in treatment we examined, only one, adjuvant chemotherapy for breast cancer, has been the subject of a consensus development conference. Thus, these conferences are not routinely used for the purpose of identifying new cancer treatments as ready for general application. However, the results of any relevant NIH consensus development conference are quickly incorporated into the PDQ statements on treatment.

Coordination Problems Exist

Our experience in analyzing the breakthroughs listed by NCI indicates that there are some problems in coordinating the agency's position on treatment advances. We were told that the PDQ statements represent the official consensus of the agency on the status of treatments. These PDQ statements indicate when a treatment is still investigational—by our definition, not yet a proven breakthrough. Yet, when we requested a list of breakthroughs that met our definition from the office of the director, the list did not reflect the PDQ positions.

The example of colon cancer therapy illustrates the problems created by the failure to coordinate the agency's positions. When we originally requested a list of breakthroughs in cancer treatment, NCI included adjuvant chemotherapy for colon cancer as one of those breakthroughs. In the course of background research, however, we had difficulty identifying any evidence within the time frame specified that supported the conclusion that chemotherapy extends survival for this disease. We contacted NCI and asked whether they were sure a breakthrough had in fact taken place. Responding affirmatively, an NCI official cited a series of clinical trials run by the Veterans Administration in the 1970's as evidence of a breakthrough occurring during the time frame we had requested. However, our check of the published trial results revealed

that these experiments had shown "suggestive" but "not statistically significant evidence" for "a marginal improvement" in survival through the use of chemotherapy.[2]

Given NCI's identification of chemotherapy for colon cancer as a recommended treatment, we retained it as one of the advances to be analyzed. Yet upon publication of our January 1988 report, the director of NCI's division of cancer treatment criticized its inclusion in our analyses. Moreover, in the March 1988 issue of the Journal of the National Cancer Institute, the editor (NCI's director of the cancer therapy evaluation program) wrote that "the lack of consensus in colon cancer is a correct reflection of the present state of our knowledge." Indeed, we have heard from researchers and oncologists in many parts of the country who questioned our identification of colon cancer chemotherapy as a breakthrough treatment.

When we consulted the PDQ system to see what information NCI was giving physicians, we learned that there are two "standard" treatments for colon cancer patients to whom the therapy should apply:

- "wide surgical resection and anastomosis" (surgery alone), or
- "wide surgical resection and anastomosis with established active adjuvant chemotherapy which is currently available only through participation in a clinical trial" (surgery followed by adjuvant chemotherapy in a clinical trial).

Based on NCI's nomination of chemotherapy for colon cancer and the PDQ statement that chemotherapy for colon cancer is available only in a clinical trial, what can reasonably be concluded about whether there has or has not been a demonstrated breakthrough in colon cancer treatment? (It would appear that in this case the very meaning of "breakthrough" can be questioned.)

We believe that the identification step we have described is complex and in need of carefully worked out criteria for determining what evidence is required to redefine an experimental therapy as a recommended one. We found that NCI appears not to fully coordinate its position on when a therapy is ready for widespread (non-investigational) use. As a consequence, it is possible that a physician looking to NCI for advice on state-

[2]P. V. Woolley et al., "Ongoing Trials in the Surgical Adjuvant Management of Colorectal Cancer," Recent Results in Cancer Research, 68 (1979), pp. 231-235.

of-the-art treatment may get many responses, or conflicting responses, depending on what office or data base is queried.

Some officials at NCI believe that this situation does not pose any problem. They assert that the agency's responsibility is to conduct the research and publish the findings that the medical community as a whole should then evaluate and reach consensus on.

To its credit, NCI currently takes positions on the merits of scientific findings by offering physicians information through the PDQ system that carries the label of "state of the art." We recognize that setting procedures for developing a consensus will not necessarily ensure that consensus is achieved. Disputes over scientific evidence will inevitably occur. What such procedures can ensure, however, is that the agency recognizes when consensus is lacking and thus when a treatment advance is not ready to be recommended for widespread use.

Diffusion

In the next step of the process—diffusion—a therapy has been recommended and the task is to convince physicians and patients of its utility. The diffusion process is the spread of a new idea—here, a treatment advance—from its source to its ultimate users or adopters—in this case, physicians. We examined what activities NCI undertakes to increase utilization of state-of-the-art treatments. Our examination focused on activities to promote adoption by physicians.

The National Cancer Act of 1971 (Public Law 92-218) required NCI to collect, analyze, and disseminate all data useful in the treatment of cancer. This included establishing an international cancer research data bank to disseminate the latest research findings.

NCI is thus required by law to disseminate information about advances in treatment, but this is only one component of diffusion. The agency is not required to actively promote the utilization of particular therapies by physicians in their treatment of cancer patients.

NCI's Role

Based on interviews and information collected from NCI staff with responsibilities in this area, we discovered that NCI supports or undertakes many activities related to the diffusion of new therapies. We found, however, that there is little agreement at NCI about what the agency's role in diffusion ought to be. Indeed, many NCI staff and directors expressed the belief that diffusion of treatments into practice is not, and should not be, NCI's responsibility. This is certainly not a new viewpoint and can be traced back as far as 1937, when NCI was created. Individuals on the National Advisory Cancer Council, who determined the course of the newly created institute, agreed that its focus should be on basic biological research and that it should not emphasize public education or patient treatment.[1]

Those we interviewed also expressed a more practical concern about whether NCI actually could influence medical practice, if in fact it were supposed to do so. Two reasons were cited to explain why efforts aimed at diffusion were not likely to succeed: the resistance by physicians to anything resembling oversight, and the fact that NCI does not regulate, license, or pay physicians. In addition, the opinion was expressed that physicians are slow to change and that little could be done to alter this. As evidence of the natural conservatism of the medical community, the

[1]J. T. Patterson, The Dread Disease: Cancer and Modern American Culture, Cambridge, Mass.: Harvard University Press, 1987, p. 131.

director of the division of cancer treatment told us that it usually takes from 3 to 10 years for a new treatment approach to be widely adopted. Whether or not these perceptions concerning physician practice are accurate, they form the context within which NCI's leadership structures its diffusion efforts.

Information Dissemination

NCI does, in fact, disseminate information on treatment advances, and we examined their efforts in this regard (a list of these activities is provided in appendix II). NCI officials noted that they always expect the results of the research they sponsor to be published in professional journals and presented at professional meetings. They cite this as a major mechanism for disseminating information. The focus for this report, however, was the activities that NCI directly undertakes and controls. These efforts include publications, the Community Clinical Oncology Program, and the PDQ data base.

Publications

Close to 12,500 subscribers receive Cancergrams (a series of monthly bulletins with abstracts of recent journal articles, books, doctoral theses, and meeting presentations), but this number translates to only about 190 subscribers for each of the 66 separate topic areas covered. Only 21 of the topics are clinically oriented and thus likely to contain information on advances in cancer therapies. Another publication, Oncology Overviews, reaches a much smaller audience. These specialized bibliographies of recent publications on a cancer topic are published irregularly and sold individually. Of the 19 issues published in 1987, sales averaged 77 copies per issue. Clearly, these publications are not reaching very many physicians.

In March 1988, NCI launched a new bimonthly Journal of the National Cancer Institute to encompass and replace the former Journal and Cancer Treatment Reports. Editorial policy aims to cover a broad spectrum of cancer research. Combined subscriber lists, deleting duplicates, totaled approximately 4,900 when the two previous journals were merged in March, but had declined to about 4,200 by late June 1988, during the transitional phase. Individuals responsible for publications at NCI commented on difficulties in marketing materials through the Government Printing Office and on funding constraints limiting in-house marketing.

Community Clinical Oncology Program

Aside from its publications, NCI has also mounted programs whose goals include the dissemination of state-of-the-art treatments. The Community Clinical Oncology Program involves community physicians in national clinical trials. One of the goals of the program is to diffuse up-to-date cancer management into the community. However, in its own evaluation of the program, NCI found no evidence of diffusion over the 3-year period studied. Patients at participating hospitals did not receive more appropriate care than patients at comparison non-participating hospitals.

PDQ Data Base

Perhaps the most cited effort at dissemination in the cancer area is NCI's Physician Data Query (PDQ) data base. The stated goals of PDQ are to

- promote diffusion of information about the treatment of cancer throughout the country,
- aid access to clinical trials, and
- increase the practical application of advances in research.

The PDQ system features an interactive computer data base that provides information about state-of-the-art cancer treatment and is updated monthly by an editorial board. It also includes a file of active cancer-research protocols and a directory of physicians and organizations providing cancer care. This directory includes some 12,000 names of practicing physicians who attest annually that they devote a major portion of their practice to the treatment of cancer patients.

The PDQ system can be accessed on-line through the National Library of Medicine, or through two private vendors. It was designed for "user-friendly" access 24 hours a day by physicians and researchers through time-sharing computer systems. New marketing techniques are aimed at expanding the ways of accessing the system. For example, three private vendors are marketing PDQ on CD-ROM (compact disk, read-only-memory), which allows the user to avoid fees for connect-time through an on-line computer system. In addition, recently developed software can be purchased to simplify access from a personal computer to PDQ on-line at the National Library. The nationwide network of the Cancer Information Service will provide PDQ runs for patients and the public who call in. However, to date, physicians have been limited to one search each through the Cancer Information Service.

Despite the seeming attractiveness of an easily accessible information system that tells physicians what treatments are recommended, 4 years

after PDQ's inception, the system is still not used very much. Use of the system has been steadily growing, but the figures are still relatively modest. Current use of PDQ through the National Library of Medicine averages 650 hours of connect time per month by about 575 users nationwide. However, NCI estimates that only about 50 to 60 percent of this time (325 to 390 hours per month) is by or for physicians. In addition to this use, about 120 hours of connect time per month is provided by vendors, all of which NCI presumes is for physicians. These figures include all institutional (hospital) use, which NCI assumes medical librarians perform at the request of a physician. The agency has no actual figures on physician use of PDQ but acknowledges that overall use of the system is low. Thus, of the thousands of physicians in the United States who treat cancer patients, including the 12,000 physicians in the PDQ directory, very few now consult the PDQ system during the average month.

Improved Marketing Efforts

Some ideas currently under consideration, or just underway, focus on improved marketing of NCI's current materials and data bases. These include using drug company representatives to distribute brochures on NCI publications to doctors, targeting mailing lists to specific user groups, and developing a PDQ marketing plan that proposes new channels for promoting physician use. It is too early to judge the effectiveness of any of these efforts. It is also important to note that NCI is evaluating many of its dissemination programs to find out if they work. These evaluations, if well conducted, should provide valuable information on how to reach physicians.

Adoption and Implementation

Informing physicians of what the new treatments are is a critical step in moving breakthrough therapies from clinical trials to the patient. However, no matter how well diffusion is carried out, the treatment will not reach the patient if the doctor does not adopt it. The best evidence to date that there is a problem with the adoption of new cancer therapies is contained in the report we issued in January of this year.[1]

In that report, we addressed the issue of the extent to which cancer patients receive state-of-the-art therapies. As we reported, we determined that use had increased for some types of treatments since 1975. Examples include radiation therapy for rectum cancer and chemotherapy for breast cancer. However, we also found that, as of the last year for which we had data, 1985, a considerable group of patients eligible for the seven therapies we examined did not receive what NCI considers state-of-the-art or breakthrough treatments. For example, 20 percent of those with Hodgkin's disease, 25 percent of those with small-cell lung cancer, and 37 percent of breast cancer patients did not receive what NCI had told us was the most appropriate treatment. Thus, there are many patients who are not receiving what NCI has characterized as state-of-the-art therapies.

In addition, even if the decision is made to use the therapy, there remains the question of whether the treatment is implemented correctly. As cancer therapies have become more complex, involving multiple treatment steps with different modalities (e.g., surgery, radiation, chemotherapy), there are more opportunities for variations in treatment to occur. With respect to the issues of correct implementation, there is only anecdotal evidence that problems may exist. For example, with chemotherapy there are questions of whether correct dosages are given at the right intervals to the right kinds of patients. Important questions remain about how soon, how frequently, and for how long chemotherapy is administered.

No study of how cancer patients are treated has been detailed enough to determine whether problems with implementation exist. It should be noted that one possible reason for the lack of such a study is the cost associated with collecting the information at the level of specificity necessary to reach conclusions.

Our forthcoming report will not directly address implementation. It will examine the impact that breakthroughs in cancer treatments have had

[1] Breakthrough Treatments for Seven Types of Cancer (GAO/PEMD-88-12BR).

on the survival rate of the cancer patient population. However, the extent to which utilization of breakthrough therapies results in gains in survival is related to the implementation of the treatments. For example, chemotherapy may require specific drugs, in specific dosages, for specific intervals to attain the expected benefits in survival. To date, no study of how cancer patients are treated has been detailed enough to determine whether problems with implementation exist.

Summary

Our evaluation of the activities of the National Cancer Institute suggest that more effort is needed in conceptualizing, defining, and establishing a process by which new therapies move from experimental to widespread use. Problems exist at many different points on the continuum along which breakthrough treatments must pass to reach cancer patients. There is a problem in coordinating the agency's positions on recommended treatments. Efforts at diffusing new treatments are limited in the extent to which they reach physicians. And in many cases, recommended treatments have not been adopted.

Without question, the role that NCI can play in increasing the utilization of treatment advances is limited if the agency does not clearly identify what it believes to be the state of the art. This is an essential first step.

Any further influence that the agency can have depends on how its diffusion role is interpreted. NCI is required by law to disseminate information, but it is not required to undertake any further diffusion activities. NCI's current dissemination activities seem to be reaching very few physicians. Unless more specific responsibilities are defined for the agency, it appears unlikely that activities aimed at promoting the use of particular therapies will be undertaken.

Matter for Congressional Consideration

Congressional committees that are concerned about efforts to promote the adoption of cancer treatment advances may want to consider steps to specifically mandate federal action. This could include directing NCI, or some other office created for this purpose, to undertake concerted efforts to clearly identify and promote the use of cancer therapies that have been sufficiently proven to warrant being classified as recommended treatments.

Request Letter

U.S. HOUSE OF REPRESENTATIVES
COMMITTEE ON ENERGY AND COMMERCE
SUBCOMMITTEE ON HEALTH AND THE ENVIRONMENT
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WASHINGTON, DC 20515
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June 2, 1987

Honorable Charles A. Bowsher
Comptroller General
U.S. General Accounting Office
441 G Street, N.W.
Washington, D.C. 20548

Dear Mr. Bowsher:

I have recently read the General Accounting Office's (GAO) report on cancer patient survival and feel that it raises some important questions regarding the future progress of cancer control in this country. In discussions between my Subcommittee staff and staff from your Program Evaluation and Methodology Division, it was mentioned that many of the experts who participated in the study felt that the impact of cancer could be reduced if available treatments were applied more widely. This position is also taken by the National Cancer Institute, which claims that as many as 40,000 cancer patients may die prematurely as a result of suboptimal care. I would appreciate your undertaking additional work that would focus directly on this potential problem.

The general objective of your work should be to determine whether the problem of suboptimal cancer care actually exists and, if so, how large it is. Specifically, I would like GAO to develop estimates of the number of patients who may die prematurely from each of the most prevalent forms of cancer because they do not receive the latest available therapies. In addition, please identify the typical length of time that passes before such therapies are adopted by community physicians and oncologists, and assess the adequacy of efforts by the National Cancer Institute to promote the use of optimal therapies.

With every good wish, I am,

Sincerely,

Henry A. Waxman

HENRY A. WAXMAN
Chairman, Subcommittee on
Health and the Environment

HAW/rfn

NCI Activities for Dissemination of Treatment Information

Listed below are activities of the National Cancer Institute aimed at disseminating information on cancer treatments to physicians. Not included are activities intended primarily to inform cancer patients or the general public.

Publications

Journal of the National Cancer Institute

In March 1988, NCI published the first issue of its new Journal of the National Cancer Institute, which combined the previous Journal with Cancer Treatment Reports. This new journal provides a peer-reviewed forum for the rapid publication of results from all areas of cancer research. Its intended audience is oncologists and oncology researchers. The combined subscriber lists of the previous two journals, totaling about 4,900 after duplicate subscriptions were deleted, had declined to about 4,200 by late June 1988, during the transition phase. In addition, about 700 copies are distributed in microfiche through the Federal Depository Library program.

Cancergrams

This is a series of monthly bulletins in 66 separate topic areas. Each issue of Cancergrams contains abstracts of recent journal articles, books, doctoral theses, and meeting presentations in the area of interest. Subscriptions to Cancergrams total about 12,500, or about 190 subscribers to each of the 66 separate series. Of the 66 issues, one series of 21 titles is on clinical topics, while two other series are in basic research areas. Roughly 125 Federal Depository Libraries received each series in microfiche in fiscal year 1988.

Oncology Overviews

Oncology Overviews are specialized, edited bibliographies of recent publications on a cancer topic. They are published irregularly, averaging 15 issues per year, 5 of which are on clinical topics. Copies are sold individually. Of the 19 issues published in 1987, sales averaged 77 copies per issue. About 125 Federal Depository Libraries receive these documents in microfiche.

Recent Reviews

Three volumes of Recent Reviews are published annually on varying topics; one volume each year is devoted to a clinical area. Each volume contains abstracts of review articles and serves as a supplement to the

Cancergrams series. Copies are sold individually. Roughly 125 Federal Depository Libraries receive Recent Reviews in microfiche.

NCI Monographs

Up to six issues of this supplementary publication are printed annually. NCI Monographs typically report the proceedings of a key conference or meeting, including relevant National Institutes of Health (NIH) consensus development conferences. Copies are sold individually. Figures on sales were not available. About 500 Federal Depository Libraries received paper copies.

Data Bases

Physician Data Query (PDQ)

The PDQ system is a computer data base, begun in March 1984, that provides the user with an interactive ability to search and retrieve cancer information. It is intended to promote diffusion of information about the treatment of cancer throughout the country, aid access to clinical trials, and increase the practical application of advances in research. The PDQ system provides information about state-of-the-art cancer treatment in a menu format. This information is updated monthly by an editorial board. It also includes a file of active cancer research protocols and a directory of physicians and organizations providing cancer care, which includes some 12,000 names of practicing physicians.

PDQ can be accessed through the National Library of Medicine's MEDLARS computer system or through certain commercial data base vendors. Automated log-on software, "PDQ Access," is available for purchase to simplify access for PDQ users at the National Library. Current usage of the PDQ system through the National Library averages 650 hours of connect time per month by about 575 users nationwide. NCI estimates that about 50 to 60 percent of this time (325 to 390 hours per month) is used by or for physicians. This figure includes all institutional (hospital) use, which NCI assumes is performed at the request of a physician. In addition to this use, vendors deliver an average of 120 hours of connect time per month, all of which NCI presumes to be for physicians. Physicians who use PDQ through the National Library's computer can obtain continuing medical education credits for the amount of time they use the system.

CANCERLIT

This comprehensive data base of abstracts of published cancer literature contains about 600,000 citations. It is updated monthly, and up to 40 percent of the entries are on clinically relevant material. CANCERLIT can be accessed through the MEDLARS computer system and through some commercial vendors. It was set up for use by medical librarians trained in the system, but there are recent efforts to make it more user-friendly. Current usage of CANCERLIT averages about 470 hours of connect time per month.

CLINPROT

The CLINPROT data base contains about 6,000 descriptions of cancer clinical trials, including U.S. and foreign protocols. About 4,000 of these entries are for completed trials, so this system serves as an archive. It can be accessed through MEDLARS by trained medical librarians. Current usage averages about 40 hours of connect time per month.

Other Programs

NIH Consensus Development Conferences

These conferences are intended for precisely the function that their title implies: to achieve consensus in an area where there has been a scientific dispute. The consensus statements resulting from these conferences are widely publicized and thus may serve to inform physicians of advances in treatment. A recent NIH-funded evaluation concluded that the conferences mostly failed to stimulate change in physician practice, despite moderate success in reaching the appropriate target audience.

NCI has sponsored or cosponsored 17 consensus development conferences, proposing one or two new topics per year, since NIH instituted them in 1977. However, only one of the breakthroughs NCI listed for us (adjuvant chemotherapy for breast cancer) has been the topic of (two) consensus development conferences.

Community Clinical Oncology Program

The Community Clinical Oncology program, which involves community physicians in national clinical trials, has the involvement of more patients in clinical trials as its primary goal. This program was developed because about 80 percent of all cancer patients are treated in their communities, rather than at major cancer centers. A secondary goal was to diffuse up-to-date cancer management into the community by involving a wider pool of practicing community physicians. It was hoped that

participating physicians would apply the cancer management principles to all their patients. It was also hoped that these physicians would, in turn, influence other physicians in the community.

The hoped-for diffusion was to be a by-product of the normal program activities. NCI took no specific actions to promote diffusion in this program. No treatment breakthroughs were targeted for general promotion: whichever protocols the participants were involved in were to serve as the basis for diffusion.

In its own evaluation of the program, NCI found no evidence of diffusion during the 3-year period studied. Patients at participating hospitals did not receive more appropriate care than patients at other, comparison hospitals.

Conference Grants

Each year NCI gives small grants to support national and international meetings and workshops at which current research results are aired and future research needs are developed. In fiscal years 1986 and 1987, respectively, 35 and 56 meetings were assisted. The total amount of funding for the program grew from about $400,000 to about $515,000 for the same years. Only a minority of these conferences involved topics covering treatments that are ready for use, however. Only 11 of 35 conferences supported in fiscal year 1986 and 10 of 56 conferences supported in fiscal year 1987 were on topics that were clearly clinical in nature. In each year, approximately 25 percent of the funding for conference grants was devoted to treatment topics.

Tumor Boards Initiative

NCI is currently administering two grants to support experiments enhancing the educational effectiveness of "tumor boards" or "tumor conferences" in U.S. hospitals. Tumor boards are multidisciplinary panels, conducted locally in hospitals, that consider patient cases. An NCI survey undertaken in 1985-1986 concluded that the patient-focused tumor boards are the most extensive professional education program available to physicians. Hospitals are required to have tumor boards to gain accreditation by the American College of Surgeons.

The two grant projects will evaluate the effectiveness of tumor boards as currently constituted in a sample of hospitals in southern California and Colorado. In addition, each project will attempt interventions that feature the increased use of PDQ. Results are not yet available from this work.

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