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CANCER PATIENT SURVIVAL: WHAT PROGRESS HAS BEEN MADE?

Statement of
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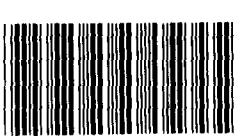
Before the
Subcommittee on Health and the Environment
Committee on Energy and Commerce
House of Representatives



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MR. CHAIRMAN AND MEMBERS OF THE SUBCOMMITTEE:

In 1985, approximately 462,000 persons died from cancer in the United States, and 910,000 new cases of cancer were diagnosed. More than $2 billion is spent annually on efforts to combat cancer with more than half this amount coming from federal funds. Given the magnitude of the cancer problem and the resources devoted to its control, it is understandable that there is considerable interest in how well efforts to control cancer are proceeding. It is a pleasure to be here this morning to share with you the results of our recently completed work addressing one aspect of this question.[1]

Before turning to our findings, I think it important to place our study in context. Progress in controlling cancer, or any other potentially fatal disease, can occur along a number of dimensions. Some of these, such as advances in basic research, are difficult, if not impossible, to quantify. However, three dimensions of progress against cancer <u>are</u> amenable to measurement. Typically, one looks to these dimensions to determine how well cancer is being controlled. One of these

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[1]U.S. General Accounting Office, <u>Cancer Patient Survival: What Progress Has Been Made?</u> GAO-PEMD-87-13 (Washington, D.C.: March 1987).

dimensions is the rate at which new cases of cancer occur in the population; this is called the incidence rate. A second dimension is the rate at which people die from cancer, and this is called the mortality rate. Finally, there is the dimension of cancer patient survival, which is usually measured by the survival rate. It is the survival rate that served as the central focus for our review.

Since 1950, the only dimension for which improvement has been reported is survival. Whereas in 1950 only one of three cancer patients lived for 5 years, by 1982, one of every two patients did so. However, a controversy has arisen as to whether the reported improvement in survival rates reflects real advances in the detection and treatment of cancer or is merely an artifact arising from the manner in which survival rates are measured.[2] The general objective of our study was to determine which of these two positions is correct. We began by evaluating the statistic at the center of the controversy--the survival rate--from a technical perspective.

The Survival Rate as an Indicator of Progress

How much do survival rates tell us about progress in

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[2]See James E. Enstrom and Donald F. Austin, "Interpreting Cancer Survival Rates," Science, 195 (March 1977), 847-51, and Richard Doll and Richard Peto, "The Causes of Cancer: Quantitative Estimates of the Avoidable Risks of Cancer in the U.S.," Journal of the National Cancer Institute, 66:6 (June 1981), 1270-81.

controlling cancer? To answer this question, we needed to determine, first, the accuracy of the rates the National Cancer Institute (NCI) publishes and second the precise meaning of survival rates as a measure of progress. To do this we

-- documented the procedures NCI uses for collecting data and computing survival rates,
-- identified actual and potential problems with those procedures, and
-- evaluated the utility of survival rates as a measure of progress in light of these problems.

To document NCI's procedures we reviewed the "methods" sections of published reports on patient survival and we interviewed NCI officials. We also concentrated on the equation NCI uses to compute its survival rates. To identify actual and potential problems with those procedures, we made an extensive review of the literature. We supplemented this review with discussions with critics to ensure that their views were well understood and appropriately represented. For our final step we compared the information on the procedures and the problems to reach conclusions about the overall accuracy and meaningfulness of the published rates.

We found that the accuracy of survival rates seems to have improved with NCI's introduction of the surveillance,

epidemiology, and end results (SEER) program in 1972. However, we also found that survival rates provide information on only one of the many objectives in cancer control--namely, extending patient survival. Survival rates do not convey much information about rates of cure or how long patients live, and provide no insight into their quality of life. What the rates do tell us is the percentage of patients who live for a specified length of time (usually 5 years) after diagnosis or, in other words, they tell us the probability that any single patient will live for that length of time.

We found, however, that changes in survival rates cannot be taken at face value. In fact, changes in survival rates are quite difficult to interpret, primarily because of continuing changes in detection practices and in what is, or is not, called cancer. These latter changes introduce a number of biases that can artificially inflate any real improvement in patient survival. We illustrate this with two examples.

A commonly accepted principle of cancer treatment is that early detection is beneficial because it allows therapy to be given early in the progression of the disease, when therapy is most effective. However, early diagnosis can also have another consequence. Let us assume that there is usually a 10-year interval between the onset of a hypothetical type of cancer and a patient's death. Let us further assume that most patients with

this hypothetical cancer typically wait until very late in that 10-year period before seeking medical attention. The 5-year survival rate for this cancer would, therefore, be low. Suppose now that a program is begun that encourages frequent checkups and, as a result, many cancers are detected much earlier than they used to be. For patients participating in this program, the 5-year survival would be considerably greater since they would generally live longer from the time they were diagnosed.

At first glance, it would appear that survival rates had improved considerably, in that patients used to live for a short period and now live for a longer time. However, this appearance is deceiving, because it equates the point at which the cancer is discovered with the point at which it actually starts. When this is done, any shift in the time of diagnosis changes the survival rate, even when there is no real change in the interval between onset of the disease and death. This phenomenon, in which patients seem to live longer but actually do not, is commonly referred to as lead-time bias.

Changes can occur not only in when cancers are diagnosed but also in the type of cancers that are diagnosed. These changes are important, because not all cancers grow at the same rate and, even within the same disease type (for example, prostate cancer), the length of time that passes before symptoms become apparent can vary. This variation means that changes in detection

practices could result in comparisons of the survival of patients with different types of diseases. For example, if all cancers discovered in 1950 were cancers that grow quickly and most cancers detected in 1960 grow slowly, the survival rate would improve even if no advances were made in treatment. This kind of problem is commonly called length-time bias.

Length-time bias is especially a problem when mass screening programs are introduced. When no widespread screening takes place, cancer is usually diagnosed after the patient or physician notices some symptom . The purpose of screening, however, is to diagnose cancers in patients who have not yet exhibited symptoms. Cancers diagnosed through mass screening may differ considerably from those of patients with symptoms at the time of diagnosis, in that some cancers may never progress to a symptomatic stage or may do so only after an extended time. Increasing the number of asymptomatic cases relative to those that already exhibit symptoms, would improve the survival rates simply as a result of length-time bias--that is, a change in what is being counted as cancer.

Because the NCI does not control for the methodological problems introduced by lead-time, length-time, and other forms of bias discussed in our report, published survival rates can present a misleading picture of progress in extending survival. Therefore, we recommended in our report that the secretary of the

Department of Health and Human Services (HHS) include in all future publications on patient survival a description of the biases that can lead to the misinterpretation of survival rate changes. HHS has concurred with this recommendation and will implement it beginning in this calendar year.

What Progress Has Been Made?

The inability to reach conclusions about what is real and what is apparent in reported survival rates raises the question of whether the lives of cancer patients have in fact been extended. To answer this question, we focused on 12 specific forms of cancer, employing a method known as information synthesis. We wanted to focus on the diseases that affect the largest number of patients, so we selected all the cancers that were among the top 10 in terms of reported incidence rates in either 1950 or 1982. This yielded 12 cancers: bladder, breast, cervix, colon, lung, endometrial, head and neck, prostate, rectum, and stomach, as well as leukemia and non-Hodgkin's lymphoma. We did not look at a number of cancers, such as Hodgkin's disease and testicular cancer, for which NCI reported that progress has been considerable. At the same time, by focusing on the most prevalent diseases we also excluded cancers of the esophagus and pancreas and some others, for which little or no progress has been reported.

Our starting point for the information synthesis was our examination of the published survival rates for the 12 cancers we selected. For each one, we learned that improvements were reported for the period 1950 to 1982. Our next step was to determine whether there was any other evidence that could support the reported improvements. To complete this step, we addressed three issues.

The first issue was whether the reported improvements in survival rates were consistent with incidence and mortality trends. As we explain in our report, the number of people who get cancer, the number who die of cancer and the length of time that cancer patients survive are logically related.[3] Because of this relationship, one can examine trends in incidence and mortality rates and then determine cancer by cancer whether changes in patient survival rates are consistent with these trends. When we found consistency between survival rates and the two other statistics, we had greater confidence that the trends reported for survival reflected at least some actual change than we did when such consistency did not exist.

The second issue was whether there was any medical reason to

3This relationship is one in which the survival rate should move in the same direction as the ratio of incidence to mortality. For example, if more people get cancer and fewer people die from it, we would expect survival rates to improve, whereas any change in survival would be inconsistent with stable incidence and mortality rates.

assume that an improvement in survival rates should have occurred. That is, were there any changes between 1950 and 1982 in the way the 12 cancers were diagnosed and treated that could have improved patient survival? Such changes would increase our confidence that at least some part of the reported improvement in survival was real, whereas their absence would increase the likelihood that the improvement was only a seeming one.

The third issue was whether the 12 cancers are prone to the types of measurement bias I mentioned earlier. For reasons that are explained in greater detail in our report, addressing this issue required us to pay close attention to changes in disease detection, staging techniques, and in the diagnosis of cancer. When we found that change had taken place in one or more of these areas, we concluded that measurement bias might turn up in the reported survival rates.

Since either "yes" or "no" was a possible answer for each of these three questions--Were the data consistent? Did the management of the disease change? Was bias possible?--eight combinations of answers were possible for each specific cancer.[4] The decision rule we used to determine whether survival had actually improved in each of these situations was a simple one. If two or more (the majority) of these dimensions supported the position that patient survival had improved, we concluded that it

[4]Yes-yes-yes, yes-yes-no, yes-no-yes, no-yes-yes, and so on.

had. Conversely, if the majority of the dimensions indicated that no improvements in survival had taken place, we concluded that none had.

We found that advances in the detection and treatment of cancer from 1950 to 1982 extended patient survival in all but 1 of 12 cancers we examined. However, we also determined that the actual improvement for specific cancers is in question, primarily because the biases we described earlier can artificially inflate the amount of "true" progress, and because NCI does not control for these biases in its published rates.

We found, too, that the improvements in patient survival have been most dramatic for the rarer forms of cancer and least dramatic for the more prevalent cancers. As a result, even though the absolute number of lives extended is considerable, this number remains small relative to all cancer patients.

With respect to what has happened in the area of patient survival, our findings indicate that both sides of the controversy can be supported, depending on the perspective one adopts. That is, while it is clear that many cancer patients have had their lives extended, their number seems small compared to the number of all cancer patients combined. The answer to the question of how well the nation is doing in efforts to control cancer, is therefore dependent on whether one emphasizes the

absolute number of patients whose lives have been extended or the percentage of patients who have benefited from advances in diagnosis, treatment, and the like. From an absolute perspective, progress has been considerable. From a relative perspective, we must conclude that not much progress has been made.

Summary

In summary, the following conclusions can be drawn.

-- Changes in when and how cancer is detected can lead to improvements in patient survival rates, even when patients do not actually live any longer.

-- The presentation of survival rates, therefore, should include a description of the biases likely to cloud their interpretation.

-- Survival rates should not serve as the sole basis for concluding that progress has or has not been made.

-- When the available evidence is assessed, it is clear that patients' lives have been extended for 11 of the 12 cancers we examined.

-- However, because the advances have been greatest for the

rarer forms of cancer, the percentage of cancer patients who now live longer is relatively small.

This concludes my remarks. I will be happy to answer any questions you may have.