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Health, Education and Human Services Division

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October 14, 1997

The Honorable Connie Mack
The Honorable John Rockefeller
United States Senate

Subject: Cancer Clinical Trials: Medicare Reimbursement Denials

In order to determine whether new drugs are safe and effective, it is essential to conduct studies in which the drugs are given to patients and the results are monitored. These studies, known as "clinical trials," can examine the effects of new (not yet approved) drugs or can test already approved drugs against new conditions. Aside from their contribution to our understanding of the best way to manage disease, clinical trials sometimes offer critically ill patients access to potentially helpful yet currently unavailable therapies.

However, many costs are associated with clinical trials, and who should pay these costs is an issue of growing concern. The costs of clinical trials include those for the drugs and for the research (that is, the collection and analysis of data) and the costs of patient care (for example, the salaries of the physicians or the expenses involved in a hospital stay if one is necessary). Although there is agreement that the sponsors of the research should pay for the former costs, debate continues about whether health insurers should pay for the "routine" costs of care. Insurers argue that because they do not typically pay for "experimental" treatments, they should not pay any of the costs associated with clinical trials. Patient advocates argue that insurers should be required to pay for the routine care that patients receive while in a clinical trial because, although the drug may be experimental, the supportive care that accompanies it often is not. Another rationale given for why insurers should pay is that many of the costs of routine care would be incurred even if patients were treated with conventional therapy. (For example, the antinausea drug given to a cancer patient receiving an experimental form of chemotherapy would also be given to patients receiving standard chemotherapy.)

It is currently HCFA's policy that Medicare will not reimburse routine patient care costs for beneficiaries enrolled in a clinical trial. The Medicare Cancer

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Clinical Trial Coverage Act (S. 381), introduced in February 1997, would establish a demonstration project requiring the Health Care Financing Administration (HCFA) to cover routine patient costs for Medicare beneficiaries who are enrolled in cancer clinical trials. The intent of the legislation is to give Medicare beneficiaries who participate in the demonstration project access to cancer clinical trials and eliminate any concern they might have about reimbursement for the costs of patient care associated with those trials.

In an effort to determine the potential effect of this legislation, you asked that we attempt to quickly estimate the current rate at which Medicare carriers deny reimbursement for routine patient care costs when beneficiaries are enrolled in cancer clinical trials. (This rate is one essential component of any effort to estimate the incremental costs to HCFA of requiring reimbursement for beneficiaries enrolled in cancer clinical trials.) As agreed with your offices, we focused on a subset of all trials that would be covered by the legislation and contacted the physicians participating in those trials.[1] All physicians were asked whether they had enrolled any Medicare beneficiaries in the trial and, if so, whether any problems were experienced with reimbursement.

In summary, 186 physicians responded to our survey, approximately 55 percent of those to whom surveys were sent. Overall, 1,143 patients were admitted into the trials we asked about and, of those, 217 were Medicare patients. Among these patients, there were eight (all reported by the same physician) for whom the Medicare carrier had not made reimbursement. Detailed data on seven of those patients showed that, in each case, the services denied were chemotherapy and drugs. Further, we learned that only one of the physicians automatically excluded Medicare patients from clinical trials because of concerns over reimbursement.

While our methods did not allow us to give a precise national estimate of the rate at which reimbursement is denied for Medicare beneficiaries enrolled in cancer clinical trials, our results suggest it is relatively rare, given the populations and time period of our review. A detailed description of our methods is presented in the enclosure.

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[1]We examined only trials listed on the Physician's Data Query system maintained by the National Cancer Institute because these were the only trials that we could identify in a timely manner. We have no way of knowing what percentage of all ongoing trials are represented by those listed in this database.

In commenting on a draft of this report, HCFA staff knowledgeable about Medicare reimbursement raised three issues. First, HCFA staff were concerned that our questions and the way they were asked could be misinterpreted by respondents in a way that would mask the extent to which claims are denied. We discussed our questions and methods and pilot-tested the survey with trial physicians and remain confident that the respondents understood our questions. HCFA staff correctly noted that we did not ask respondents the extent to which they might never submit a claim for reimbursement to Medicare because of their expectation that it would be denied, per HCFA policy. To do so would have expanded our work beyond its purpose of examining only claims denials.

Second, HCFA staff commented that our response rate was inadequate, at just slightly over half of physicians surveyed. We have no reason to believe that physicians who had more difficulty with reimbursements would have been less likely to respond to the survey. The uniformity of the results also makes the findings worth reporting.

Finally, HCFA staff reported that, given our findings, their actuaries have nearly doubled their estimates of the extent to which Medicare mistakenly reimburses claims for routine patient care costs.[2] Under HCFA's current policy, any reimbursement for care associated with a cancer clinical trial would be made in error.

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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. At that time, we will send copies to interested parties and make copies

[2]HCFA initially estimated that 25 percent of the claims under Medicare Part B and 10 percent of the claims under Medicare Part A were mistakenly reimbursed. These estimates have been revised to 50 percent and 15 percent, respectively.

available to others on request. This letter was prepared by Carolyn Feis and George Silberman. Please call me at (202) 512-7119 or Dr. Feis at (202) 512-3864 if you or your staff have any questions .

Sincerely yours,

Bernice Steinhardt
Director, Health Services Quality
and Public Health Issues

Enclosure

SCOPE AND METHODOLOGY

As agreed with the requesters' offices, we defined the scope of the study so as to allow for data collection within a short time and with minimum respondent burden. Therefore, we focused our attention on a subset of all trials. The criteria we used to define that subset and their rationale are presented below.

First, because no database identifies all ongoing cancer clinical trials, we focused our attention on trials listed in the Physicians Data Query (PDQ) database maintained by the National Cancer Institute (NCI). NCI and other National Institutes of Health (NIH) studies are automatically included in PDQ, as are others. Any study, however, may be submitted for review. Approximately one-third of the active trials listed in PDQ are submitted voluntarily.

Second, in order to ensure that Medicare patients were likely to be enrolled, we focused on clinical trials of treatments for types of cancer that were most prevalent among the elderly. We selected trials that enrolled patients with breast, colon, rectal, prostate, or lung cancer.

Third, while the legislation would require Medicare carriers to reimburse patient care costs independent of the phase of the trial, we limited our review to Phase II and Phase III trials. Phase I trials are studies in which a treatment is first tested in humans. The purpose is largely to determine the safety of the treatment. Because of the risks to patients from these untested modalities, Phase I trials involve few patients, and the average enrollment per trial is very small.[3]

Fourth, we selected March 1, 1996, through September 30, 1996, as our period of study. Rapid changes in the U.S. health care system outdate information about reimbursement decisions made years ago. Therefore, we were interested in patients treated recently. However, because the ultimate decisions on reimbursement are often reached through iterative questioning and appeals, we needed to allow sufficient time for the denial decision to "mature." In conversations with physicians who participate in trials, we learned that the enrollment date was usually proximate to when treatment began and that it could take as long as 6 months to resolve requests for reimbursement. Therefore, we focused on patients enrolled between March 1 and September 30, 1996.

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[3]Phase II and III trials, in contrast, are aimed at determining the effectiveness of treatments and threfore require larger numbers of patients in order to offer reasonable chances for observing an effect, even if it is small.

Finally, we excluded trials that provided solely inpatient treatment. This decision was based on the reality that insurers rarely know what specific inpatient services are provided. Reimbursement for inpatient services is determined by a diagnosis, not by the specific treatment. Because the treatment modalities are not specified to the insurer, insurers usually cannot discriminate between experimental and conventional therapy.

After identifying active trials investigating one or more of the five cancers we were interested in, we surveyed the individuals who chaired the studies to confirm that a trial had enrolled patients during the period we were interested in, to determine the number of patients admitted to the trial, and to verify that outpatient services were provided.

Each trial could have multiple physicians listed as principal investigators. For the trials that met our criteria, we determined the average number of patients per physician listed as a principal investigator and selected those for which the average was greater than or equal to two. We did this because the likelihood of obtaining relevant data was minimal for trials with a smaller ratio. Therefore, we surveyed all principal investigators PDQ listed as associated with the trials we included.

We sent surveys to 340 different physicians working on 49 different trials and included one follow-up mailing. We completed our work between February and June 1997 in accordance with generally accepted government auditing standards. Our findings are based on surveys received from 186 doctors working on 39 different trials, representing response rates of 53 and 80 percent, respectively.

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