C L C CANCER LEADERSHIP COUNCIL

A PATIENT-CENTERED FORUM OF NATIONAL ADVOCACY ORGANIZATIONS ADDRESSING PUBLIC POLICY ISSUES IN CANCER

July 17, 2000

Health Care Financing Administration
Department of Health & Human Services
200 Independence Avenue, S.W.
Room 443-G - HHH Bldg.
Washington, D.C. 20201
ATTENTION: HCFA-3432-NOI

Re: Notice of Intent to Publish a Proposed Rule, 65 Fed. Reg. 31124 (May 16, 2000)

Dear Sir/Madam:

The undersigned groups submit these comments on behalf of people with cancer, their caregivers and cancer research organizations. We appreciate the opportunity to provide advance comment regarding the agency's plans to institute new coverage criteria for items and services provided to Medicare beneficiaries. As our comments below indicate, we have serious reservations about, and objections to, these plans, but expect to comment more extensively should the agency proceed with publication of a proposed rule.

Attributes of Modern Cancer Care

Our concerns about the proposal are based in significant part on our perception that it is inconsistent with the way quality cancer care is practiced today. Therefore, we think it appropriate to review briefly some of the characteristics of modern cancer therapy, which we believe must be taken into account before establishing new, more restrictive coverage criteria.

Treatment of cancer is highly individualized.

Cancer is not one but more than a hundred different diseases. Even among very specific cancer types, there may be great heterogeneity in different patients. Cellular characteristics of each patient's cancer may vary significantly, and treatment may correspondingly vary. In such circumstances, formulaic approaches to coverage determinations should be suspect to the extent that such determinations limit the treatment options available to individual patients with their potentially very different diseases.

Cancer research is well organized and integrated into standard patient care.

Cancer is unique among diseases in that it alone benefits from an extensive government-sponsored research network that provides patients, even in remote locations, access to new

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therapies through clinical trial participation. The research structures in cancer, under the auspices of the National Cancer Institute and otherwise, provide monitoring, evaluation and eventual publication of clinical trials results, ensuring a degree of oversight and quality perhaps not so consistently found elsewhere. The research orientation of cancer care helps to drive cancer therapies toward gradual improvements in morbidity and mortality rates, as recent national statistics have shown, and cancer advocates do not favor relinquishing decisionmaking to entities that are not involved in the cancer research enterprise.

Progress in cancer is incremental but relentless.

One seldom sees dramatic or immediate improvements in cancer therapy, but progress occurs nevertheless, as the data show. This progress depends mostly on small refinements in treatment discovered through the painstaking process of many clinical trials, testing new hypotheses and eventually establishing new standards of care, albeit often not markedly different from before. Cancer is thus a therapeutic area that must value incremental steps and decline to embrace dramatic developments as the standard for coverage.

Standard cancer therapy can change rapidly on the basis of research reports.

Even though progress against cancer is usually incremental, small changes in therapy can be incorporated quite quickly as research results become available to oncologists. Historically, the pace of change in cancer care has been such that the review processes of the Food and Drug Administration (FDA) lag behind, as do specific coverage decisions by third-party payers. Accordingly, it is well established in cancer that so-called "off-label," or not specifically approved, uses of FDA-approved drugs are appropriate for cancer therapy and should be eligible for reimbursement. Such special circumstances in cancer calls for coverage rules tailored to its particular needs.

Statutory Limitations

While any proposed coverage criteria should consider the special circumstances of cancer therapy, they must also be guided by any pertinent statutory restrictions. There are at least two such restrictions in the Medicare authorizing legislation, one very specific and the other general but nonetheless important.

Coverage standards for anti-cancer chemotherapy are set by statute and may not be altered by regulation.

During the 1980's and early 1990's, cancer patients experienced many denials of coverage for medically appropriate cancer care because their therapy involved "off-label" uses of approved drugs and such uses simply were not recognized as valid by carriers and intermediaries. In response to concerns expressed by cancer patients and their physicians, Congress mandated the Medicare program to cover all medically appropriate uses of FDA-approved drugs employed in an anti-cancer chemotherapy regimen, with medical appropriateness to be determined by reference to independent expert sources specified in the statute. See 42 U.S.C. 1395x(t)(2), enacted in the Omnibus Budget Reconciliation Act of 1993. Given this explicit statutory directive, the agency has no latitude to apply different or more restrictive coverage criteria with respect to cancer therapy involving drugs or biologicals.

The Medicare statute does not authorize "cost-effectiveness" as a basis for coverage decisions.

The Notice assumes, without discussion or justification, that the Medicare program is authorized to make coverage determinations on the basis of cost-effectiveness or cost comparisons among different technologies. It is by no means clear that this approach was intended by Congress. Certainly, if the views of those charged with implementing the statute at the outset were taken into account, there would be no reason to believe that cost should be a consideration in coverage decisions, and in fact for most of the life of the Medicare program cost has not been a factor. Only in the failed Notice of Proposed Rulemaking in January 1989—25 years after the initiation of the program—did agency officials suggest that cost-effectiveness might be considered, and of course that proposal sat unfinalized for more than a decade and was ultimately withdrawn in April 1999. The assumption that cost-effectiveness is inherent in the statutory standard requiring coverage of items and services "reasonable and necessary" for medical care will not go unchallenged. Many would argue—and the agency's own history of interpreting the statute would support—that, so long as financial resources are available (as they clearly are in an entitlement program), the reasonableness of treatment for a life-threatening disease like cancer should not depend on its cost. If the agency insists on retaining cost-effectiveness as a coverage criterion for life-threatening diseases when and if it publishes a proposed rule, we will have much more to say then with respect to the appropriateness of this approach.

Concerns of the Cancer Community

In light of the above background information on cancer care and the above-discussed statutory limitations, we urge the agency to consider the following before publishing a proposed rule implementing a new coverage process.

Abandon cost-effectiveness as a coverage criterion, at least for life-threatening diseases like cancer.

Given the lack of apparent statutory authority for application of this proposed coverage criterion and the impact it could have on development of, and access to, potentially life-extending new therapies, we urge the agency not to proceed until such time as the Congress has specified the role, if any, that cost-effectiveness should have in making coverage decisions for the Medicare program.

Clarify that the proposed coverage criteria will not apply to anti-cancer chemotherapy.

In light of specific statutory guidelines on Medicare coverage of drugs and biologicals used in an anti-cancer chemotherapy regimen, the proposed coverage criteria should explicitly exempt items and services associated with this statutorily mandated coverage

Maintain the role of informed physician decision-making in highly individualized diseases like cancer.

In diseases with great individual variability and thus some degree of uncertainty in diagnosis and treatment, rigid coverage formulas may inhibit best care. In the treatment of cancer, the individual oncologist must retain flexibility to pursue the therapy that, in his or her well-informed judgment, is necessary for the particular patient.

Define "breakthrough" technology to include incremental advances in life-threatening diseases like cancer.

As noted above, cancer treatment is characterized by small advances in outcome through often rather minor revisions to standard care determined through many clinical trials. For people with life-threatening cancer, no advance is too small to be considered a "breakthrough" as described in the agency's proposal. In this and other aspects of its adoption of new coverage criteria, the agency should remain mindful of the special circumstances that may apply in the case of potentially life-threatening diseases like cancer.

Tailor evidence reviews to correspond to the needs of the individual disease and other particular circumstances, including the need for rapid and efficient review of new therapies.

The cancer community is strongly supportive of reliance on sound clinical evidence in the determination of medical practice. However, third-party payers like Medicare must resist the temptation to use evidence review as a means of inhibiting dissemination of new technologies. Moreover, the process for determining coverage must be efficient enough to avoid delays in access to potentially beneficial therapy for people with life-threatening diseases like cancer. In general, the agency should seek to rely on existing independent resources rather than creating its own new bureaucracy for the purpose of reviewing clinical data. At least in cancer, the leadership of the National Cancer Institute and various other evidence-evaluating and guideline-setting organizations provides ample opportunity for the Medicare program to seek outside guidance regarding the safety, efficacy and medical appropriateness of new therapies. By using these resources, the program can make decisions that are informed by the best medical judgment in each particular medical specialty in the most direct and efficient manner and without the delay and expense of establishing an elaborate new review process within the government.

Do not penalize the Medicare population for lack of age-specific data.

The Notice suggests that the agency might require clinical trial evidence specific to the Medicare population in making coverage determinations. We believe this would be an inappropriate burden on beneficiaries' access to new technologies. It is well known, certainly in the cancer community, and even supported by a study of the Southwest Oncology Group, that the Medicare population is seriously under-represented in cancer clinical trials. One reason for this situation, however, is almost certainly the position of the agency with respect to coverage of routine patient care costs in clinical trials. If the agency adopts the position articulated by the President in his June 7 Executive Memorandum with respect to coverage of

patient care costs in clinical trials, this particular impediment will be removed. But there will still be many reasons why Medicare beneficiaries are not adequately represented in clinical trials, including restrictive eligibility criteria. Beneficiaries, however, should not be deprived of access to new therapies—particularly in the case of life-threatening diseases like cancer—solely because of lack of age-specific data and in the absence of any reason grounded in safety or efficacy to doubt that such therapies would be appropriate in elderly patients.

CONCLUSION

The cancer community strongly supports the practice of evidence-based medicine, in oncology and elsewhere. We also recognize, however, that a single formula for determining access to new technologies may not be appropriate, especially for disease like cancer that have many unique attributes, as discussed above. Therefore, we urge that any new coverage process be sufficiently flexible to account for the particular circumstances of different diseases and different patient populations, as Congress has already indicated with regard to anti-cancer chemotherapy coverage. In addition, any new coverage process must be efficient and timely, especially where new technologies under review are for the treatment of life-threatening diseases.

Finally, any new process should proceed with caution in employing cost-effectiveness as a means of denying coverage. The "science" of cost-effectiveness analysis is not well-defined, and the Medicare program has no meaningful experience in applying such criteria to coverage decisions. Use of cost-effectiveness as a coverage criterion should await specific authorization and guidance from Congress.

Cancer Leadership Council

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