



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

November 17, 2000

John M. Eisenberg, M.D.
Director, Agency for Healthcare Research and Quality
2101 East Jefferson Street
Executive Office Center - Suite 600
Rockville, Maryland 20852

Dear Dr. Eisenberg:

The undersigned organizations advocate for responsible public policy positions concerning cancer treatment and research. Individually and collectively, we have long urged third-party payers, including Medicare, to agree that routine patient care costs should be covered, regardless of whether such care is provided in the context of standard therapy or a clinical trial. Our position was bolstered by the fact that, at least for cancer patients, treatment in a clinical trial is considered to be consistent with best available care. Thus, when the President announced on June 7 a policy of Medicare reimbursement for routine patient care costs associated with clinical trials, we believed we were on the road to an enlightened model coverage policy.

Revisions of the coverage proposal and decisions related to the implementation of the final coverage decision have raised serious concerns in the cancer community. We strongly recommend that: 1) the clinical trials self-certification process be simplified; and 2) automatic Medicare coverage be extended to those trials that are exempt from investigational new drug application (IND) requirements under 21 C.F.R. 312.2(b)(1), or so-called IND-exempt trials.

Self-Certification Criteria

The President's decision to extend Medicare coverage to routine patient care costs in clinical trials was explicitly based on a study and formal recommendations by a prestigious expert panel of the Institute of Medicine (IOM), convened pursuant to an Act of Congress. After reviewing all the facts and law and hearing the views of numerous experts in clinical research and medicine, the IOM issued recommendations, the first of which was that "Medicare should reimburse routine care for patients in clinical trials in the same way it reimburses routine care for patients not in clinical trials."

The IOM panel made two recommendations which are relevant to the issues currently under consideration by the Agency for Healthcare Research and Quality (AHRQ). IOM recommended:

- A broad definition of clinical trials which should be eligible for reimbursement. Trials of all phases and legitimate designs and sponsorship (government, industry, or other) were included in the IOM recommendation. The basic standard set by IOM required a trial to: 1) have a written protocol that describes and scientifically sound study; and 2) be approved by all relevant Institutional Review Boards (IRBs) before participants enroll.
- No special precertification by HCFA, or any other administrative process, for researchers or physicians participating in trials. IOM proposed that claims for care in a trial should be submitted in the same manner as claims for care outside a trial. According to the IOM panel, if there is an explicit reimbursement policy for care in a clinical trial, there should be no requirement that researchers adhere to a certification process.

We endorse the broad coverage standard articulated by the IOM panel, which captures our longstanding position that routine patient care costs for those participating in a trial should be reimbursed in the same manner as costs incurred outside trials. Imposing qualifying criteria on trial sponsors and requiring them to certify their compliance may discourage sponsor, physician, and patient participation in trials. A primary objective of the President's clinical trials policy was to increase senior citizen participation in trials, but critical implementation decisions may undermine the policy.

If the Medicare program is intent on subjecting IND-exempt trials to a self-certification procedure, then it is imperative that the process be simple, straightforward and objective. There should be no opportunity for subjective second-guessing by federal officials if we are to avoid a damaging chilling effect on clinical cancer research. Thus, we strongly support the basic criteria outlined in the IOM report. According to the IOM report, the criteria for self-certification should be limited to (1) approval by an IRB and full compliance with other relevant federal requirements, and (2) use of a written protocol with defined end points. There should not be any requirement for these trials to be conducted by investigators or institutions with federal sponsorship or funding.

“Deemed” Coverage for IND-Exempt Trials

At the same time we recommend that the criteria for coverage of IND-exempt trials be simple and clear, we strongly urge the Health Care Financing Administration to reconsider the requirement that IND-exempt trials be certified for reimbursement. In contrast to the proposed coverage decision, the final version did not extend “deemed,” or automatic, coverage to clinical trials exempt from the otherwise applicable requirements of “investigational new drug” review by the Food and Drug Administration (FDA). Under 21 C.F.R. 312.2(b)(1), an IND is not required for investigations involving already approved drugs if certain criteria are met, notably if the investigation complies with IRB standards and if the investigated usage does not significantly increase risk to the patient. Essentially, these IND-exempt trials are viewed, as a class, by FDA as not requiring hands-on review, largely because they involve relatively minor deviations from the approved usage. Failing to include these trials in the category of “deemed” coverage will discourage investigator-initiated trials and undermine the original intent of the President's June 7 executive memorandum. For the following reasons, we request that the coverage decision be revised to extend “deemed” coverage to IND-exempt trials, at least those involved in cancer clinical research.

1. IND-exempt trials are an integral part of cancer care in the United States.

Thousands of cancer patients are enrolled in investigator-initiated clinical trials that are under the funding sponsorship of pharmaceutical companies or other private entities. If, as we believe, the self-certification process envisioned by Medicare for these trials acts to discourage them, then patients will be deprived of investigational treatment options otherwise available to them. While the differences between the approved usage and the investigational approaches pursued in these trials are relatively minor, such small differences can be significant in cancer therapy. It is, after all, through these incremental steps that measurable progress against cancer has been achieved.

In general, access to clinical trials is considered integral to quality cancer care. Clinical trials are so much a part of standard cancer care that 80% of cancer physicians have participated in a clinical trial in recent months, according to a survey of its members conducted by the American Society of Clinical Oncology (ASCO), the leading medical society for physicians treating cancer. One important category of these cancer clinical trials is investigator-initiated, IND-exempt trials that are disadvantaged under the final coverage decision.

2. IND-exempt trials are an important component of the overall clinical cancer research effort and should not be deterred by Medicare coverage policy.

While, by definition, IND-exempt trials are generally not intended to result in FDA-approved labeling changes for the study drugs, company and other sponsors require rigor in trial design, data collection and reporting of results. It is a fact that some significant advances in treatment--e.g., weekly administration of taxol--have emerged through investigator-initiated trials of the sort that are at issue here. Moreover, the results of these trials have been published in distinguished peer-reviewed medical journals such as the Journal of Clinical Oncology. It would be a loss to clinical cancer research and would restrict patient treatment options if the number and scope of these trials were reduced by virtue of Medicare policy, particularly in a context where the President clearly intended to support the research enterprise.

The evident intent of both the President and the IOM was to ensure that Medicare beneficiaries received all the care to which their premiums entitle them, including access to clinical trials. It would be wrong for the Medicare program to seek to use reimbursement policy--ultimately affecting quality of cancer care for beneficiaries--to exert leverage or control over appropriate private sector research activities, especially when such authority has specifically been given by Congress to the FDA.

3. The prospect of retrospective audits and reimbursement recoupment will effectively deter participation in clinical trials by physician investigators.

Comments submitted to the Health Care Financing Administration (HCFA) from various medical associations made clear that the self-certification process will deter physician participation in clinical trials. Oncologists are not exempt from the same fears and concerns that motivate other specialties. Based on the history of recent aggressive auditing and recoupment activity by HCFA and the Inspector General, physicians will no doubt think twice before making a specific, sworn representation that their activities comport with criteria that may be vague, subjective and subject to revision by federal officials.

4. Medicare coverage of routine patient care costs is, and should be, presumed, and there is no basis for overturning that presumption with respect to IND-exempt clinical trials.

It is important to remember that the issue here revolves around “routine” patient care costs--items and services like physician charges, hospital stays and standard diagnostic procedures. These costs are ones to which Medicare beneficiaries are undoubtedly entitled, and, if such costs are not reimbursed, beneficiary premiums will have been paid in vain. Thus, there is an effective presumption that such costs will be covered, and it is only when the Medicare program takes affirmative action in the form of a non-coverage decision that these costs are usually at risk of not being reimbursed.

Here, the Medicare program has made the decision that routine patient care costs should be paid when incurred in connection with government-sponsored clinical trials or clinical trials conducted under an IND. The program has thus judged that these costs should be covered even when the context of treatment is purely investigational, even involving largely unproved therapy such as drugs or biologicals that have never been approved by FDA or any other regulatory authority. It is therefore most anomalous that the program would not extend the same degree of coverage to IND-exempt trials, which involve already approved drugs being investigated in often slight variations from the FDA approval. Indeed, it is difficult to articulate a policy that would justify failing to extend coverage to IND-exempt cancer trials when such coverage is available in trials conducted under an IND.

This approach can only be viewed as a means of asserting Medicare control over purely privately funded research, despite the fact that FDA, as the principal federal regulatory authority, has made a considered policy judgment that these trials do not require that sort of oversight. The rights of Medicare beneficiaries to have their routine patient care costs covered in this setting should not be placed at risk when a clinical trial is otherwise in compliance with federal requirements.

CONCLUSION

HCFA should reissue the national coverage decision, reverting to the original proposal, which included IND-exempt trials in the “deemed” covered category. If HCFA chooses not to do so across the board, it should at least do so with respect to cancer clinical trials. If IND-exempt trials are not restored to “deemed” status, the criteria for self-certification should be simple, objective, and not subject to subjective second-guessing by federal officials.

We appreciate the opportunity to comment on implementation of this important policy.

Cancer Leadership Council

American Society of Clinical Oncology
Cancer Care, Inc.
Colorectal Cancer Network
Cure For Lymphoma Foundation
International Myeloma Foundation
Multiple Myeloma Research Foundation
National Coalition for Cancer Survivorship
National Patient Advocate Foundation
National Prostate Cancer Coalition
North American Brain Tumor Coalition
Oncology Nursing Society
Ovarian Cancer National Alliance
Pancreatic Cancer Action Network
The Susan G. Komen Breast Cancer Foundation
The Wellness Community
US-TOO International, Inc.
Y-ME National Breast Cancer Organization

cc:

The Honorable Donna Shalala
Secretary, Dept. of Health & Human Services

Michael Hash, Acting Administrator
Health Care Financing Administration