Patients Against Lymphoma

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Sunday, February 29, 2004

Mr. Dennis G. Smith
Centers for Medicare and Medicaid Services
Department of Health and Human Services
Hubert H. Humphrey Building
200 Independence Avenue, SW
Washington, DC 20201

Re: Medicare Coverage Policy That Would Deny Reimbursement for "Off-Label" Use of Some Cancer Drugs

Dear Mr. Dennis G. Smith:

Patients Against Lymphoma, representing the undersigned cancer patients, their loved ones, and the public, are writing to express a serious concern about a proposed drug coverage policy reported in the press. Apparently, the Centers of Medicare and Medicaid Services (CMS) is considering policy changes in order to reduce costs. Alarmingly, the savings would be realized by limiting "off-label use of prescription drugs", specifically that treat individuals fighting lymphomas and other life-threatening cancers.

We submit that this policy would limit a physician's ability to meet the unique and special needs of individuals who face the disease, and that this would impact millions of Americans who now have a real hope of escaping or managing cancer and living productive lives – that it would: (1) increase mortality; (2) increase pain and suffering, (3) impede innovation and progress, (4) and increase the costs and complications associated with standard treatment.

We have no choice but to take the reported statements seriously, especially as they were made by the chief health officer at CMS, Dr. Sean Tunis. We ask that you carefully reexamine the stated policy idea, and the rationale given to support it. In our view this course of action must not be allowed. We discuss the many dangers of the proposed policy change, and the weaknesses of the rationale, following excerpts from the reports:

> Federal administrators who run the Medicare program have begun to closely scrutinize the off-label use of prescription drugs, using clinical reviews to determine which prescriptions will get into the hands of American consumers and which won't. …
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> … Medicare officials said cost was the program's primary concern in reviewing its reimbursement policy for two of the drugs being studied -- Bexxar from GlaxoSmithKline and Zevalin from Biogen Idec. The drugs are approved as a third-line treatment for non-Hodgkin's lymphoma, a form of cancer that begins in the lymph system, after other treatments have failed. But they are sometimes administered as a first line of attack, a use that is not approved. The medications cost $22,210 for a single dose, which constitutes a full course of treatment, according to spokeswomen for both companies. …
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> ~ **New York Times** - Medicare Law Might Limit Drug Discounts For Insurers December 24, 2003 By Gardiner Harris

"Dr. Sean Tunis, chief health officer at CMS, said the review of cancer drugs grew partly out of a feeling that doctors were prescribing expensive medications based on "non-clinical concerns," including promotions by drug companies. …

… Tunis said he supports another form of review that could be used in the future: "head-to-head" trials within a class of similar drugs to see which brand or generic is the most cost-effective. Medicare reimbursement rates could then be pegged to the least costly of the effective drugs, he said. "One of the key gaps now is head-to-head trials, which for the most part are not required for FDA approval but which I think are increasingly important for reasons of reimbursement," he said. The approach would be similar to limitations private health insurers already place on their drug lists. …

… Meanwhile, the agency is considering revoking coverage for off-label uses for some cancer drugs, including Biogen Idec's Zevalin, an expensive treatment for non-Hodgkins lymphoma that is used off-label to treat other forms of cancer.

~ **Boston Globe** - Medicare scrutinizing off-label uses for medicine By Christopher Rowland, Globe Staff, 2/10/2004

The policy of limiting off-label use sets a dangerous and disturbing precedent that:

- **Would limit a physician's ability to select, combine, and adjust treatment plans to meet the unique and special needs of their patients.**

  Patients with cancers are different from each other and have complex clinical circumstances: age, general health, where the tumors are located, differences in tumor types, aggressiveness, other illnesses or conditions the patients may have, tolerance for the toxicities of some chemotherapy drugs, and so on.

  Patients can also have different treatment goals and priorities, such as high or low tolerance for taking risk, or the desire, or necessity, to keep working during treatment.

  We have a moral obligation to do no less than our best for individuals facing life-threatening diseases. And so it follows that only trained physicians, guided by the peer-reviewed medical literature, can be entrusted to select therapies that are appropriate and tailored to meet their needs.

- **Would deny reimbursement for frontline use of Zevalin and other expensive drugs for cancer.**

  Our group supports patients fighting against lymphoma, and we believe the frontline use of Zevalin to be very rare at this time. That said, the use of this or similar drugs could be a more reasonable choice for patients facing some subtypes of lymphoma, such as Mantle Cell Lymphoma – a cancer that does not often respond well to approved frontline chemotherapy.

- **Could limit or prevent frontline use of new biological drugs like Rituxan as single agent therapy for slow-growing lymphomas**.

  Administered intravenously, Rituxan seeks out and binds to cells in the body that have a specific protein found only on mature b-cells, and therefore on the majority of b-cell lymphomas. It is thought that this binding activates the body's own defense mechanisms to attack these cells, and that the binding may also cause the cells to self-destruct. Targeting the attack on the cancer in this way reduces many of the side effects of broad-based chemotherapy treatments, which tend to damage more healthy tissues; often creating additional problems that must be treated, in addition to the cancer.

  Targeted therapies can effectively control lymphomas, and maintain quality of life. They may be less likely to preclude future treatment options as well – leaving the patient able to benefit from standard treatments, or new treatments that may be developed in the future.

Like many cancer therapies, Rituxan won approval by demonstrating efficacy in patients with one subtype of lymphoma, who have had prior chemotherapy. But what is understood about how it works, its lack of toxic side effects, and the fact that use of toxic chemotherapy has not provided a survival benefit for patients with indolent lymphomas [1] makes the off-label frontline use of Rituxan reasonable in other settings.

- **Would be a disincentive for the pharmaceutical industry to develop new cancer drugs.**

  Developing and testing new drugs to treat people who have cancer is a difficult, time-consuming, and expensive process. Recent estimates show the cost may be as high as $900 million to get a new cancer drug to market. We need to adopt policies that will make companies more willing to take these financial risks, not less willing, if we are to make significant progress in treating the victims of cancer.

<u>The rationale in the press reports for the policy change implies:</u>

- **That head-to-head trials can answer important questions about best use of cancer drugs, and would lead to reduced costs.**

We agree that the patient community benefits from research using well-controlled studies, but the suggestion that additional head-to-head studies (post approval) will reduce costs and answer "best use" questions betrays a simplistic understanding of what is possible to do, or uses simplistic reasoning which can misleadingly influence opinion. For example:

(1) Who will pay to design and run each study for each drug pairing for each indication?

(2) Will patients enroll in the studies in sufficient numbers?

(3) Has CMS considered that requiring head-to-head studies post approval would further reduce the pool of patients eligible for clinical trials, and thus delay evaluations of emerging therapies for cancer?

(4) Will CMS mandate controlled studies for each of the 38 subtypes of lymphoma, for example?

(5) Has CMS considered the human cost – suffering and mortality - of further delaying access to new drugs while each study is conducted for each indication and each treatment setting?

(6) How will the study results in controlled populations be reconciled with real-world situations, such as the co-morbidities, tolerance for toxicities, different treatment goals and tolerance for risk, and so on?

- **That doctors prescribe off-label therapeutics based on drug company promotions or self interest - that doctors in general cannot be trusted to have their patients' best interests at heart.**

  The following reply from ANCO to a similar charge made in the press about oncologists, seems far more in line with patient experiences, and reality:

  "To the contrary, decisions about whether to undergo or continue chemotherapy treatment are driven by well-established scientific protocols and the desires of patients and their families. Cancer is an often lethal disease that the cancer community is dedicated to fighting, and part of

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[1] A systematic overview of chemotherapy effects in indolent non-Hodgkin's lymphoma. Acta Oncol. 2001;40(2-3):213-23. Review.

that fight includes attention to the quality of life of the patient. Oncologists make treatment decisions based on the best interest of the patient, not chemotherapy drug reimbursement. Sometimes, the decision is to not treat. And, patient support and satisfaction with oncologists is amongst the highest of any patient population being treated by specialists." [2]

Please note that what patients often report as the key factor influencing what cancer therapy is offered to them is the uncertainty that insurance will reimburse for the treatment judged most appropriate by his or her doctor.

We believe that no one can afford to ignore these issues, or to be silent. The numbers tell us that directly or indirectly almost every American will experience cancer. In 2003 new cases topped 1.3 million, resulting in approximately 556,500 deaths in that year alone. [3]

The rationale of the CMS official seems to be a poorly considered attempt to obscure the real motivation: That CMS policy shift is really about saving money no matter what the doctors think is best for their patients, or what additional post-approval studies may show.

We consider the precedent of this policy, or any similar policy, to be a threat to the survival of patients fighting cancer today and those who will fight it in the future. It would increase pain and suffering. It would increase mortality. It would hold back innovation and progress at a time when we are beginning to "*realize the promise of genomics and proteomics for the prevention and cure of cancer and other diseases.*" [4] It would impact the fates of Americans who now have a real hope of escaping or managing cancer and living productive lives.

It's easy to make policy decisions when the policymakers do not have to face the individuals whose lives will be affected – *possibly irreversibly* – by these decisions. Therefore, we are attaching several first-person accounts – *from real people with cancer* – about the potential benefits of using new cancer treatments off-label.

Finally, we believe that all Americans must insist that CMS work with the medical community, patient advocates, and our elected representatives, to find ***humane*** and reasonable ways to reduce the costs of health care.

We look forward to your response addressing our concerns.

Respectfully,

Karl Schwartz
President, Patients Against Lymphoma

cc: Our representative

Attachments: 6

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[2] ANCO Responds to Slanted NY Times article About Drug Reimbursement and Cancer Doctors - John A. Keech, Jr., D.O., ANCO President & José Luis González, ANCO Executive Director

[3] SEER 2003

[4] Sept 2003 - National Biospecimen Network Blueprint - representing the collaborative efforts of scientists, clinicians, industry representatives, and patient advocates who participated in the design of a National Biospecimen Network (NBN) Blueprint with the ultimate purpose of accelerating scientific discovery in the battle against cancer.