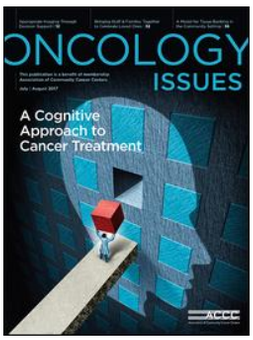

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Lee E. Mortenson

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Study Supports Widespread Off-Label Drug Use, Compendia's Ability To Impact Reimbursement

By Lee E. Mortenson, D.P.A.

In 1986, the Association surveyed the offices of medical oncologists to determine the extent of off-label use. This survey was conducted at a time when the "off-label" debate was in its infancy. At that time, a number of pharmaceutical companies assumed that the rejection of reimbursement for interferon by a significant number of insurers represented a "badly planned marketing launch," rather than a new trend in insurer coverage policies.

We conducted the original survey because of a meeting held in Los Colinas, TX, the preceding fall. During that meeting, a representative of a national insurer told us that the industry intended to force the Food and Drug Administration (FDA) to become the authority on what should and should not be reimbursed. The stated threat was that if oncologists continued to press for payment of new and expensive therapeutic agents, the insurance industry would insist on paying only for those indications on the label, and that they would extend this threat to old as well as new indications! We had no idea of the extent of off-label use in those days, so the initial survey was an impressive surprise: 47 percent of all drug use was off-label.

In the interim years, as it has become obvious that the National Blue Cross and Blue Shield Association is leading the charge to limit payments for off-label indications. We have seen extensive documentation of the problems this poses for cancer patients and their physicians. A Gallup Survey conducted last year, which was sponsored by Lederle Laboratories in conjunction with the National Cancer Institute, found that oncologists believe one out of every eight cancer patients in the United States do not receive the physician's treatment-of-choice because of reimbursement problems, most notably off-label treatments.

A U.S. General Accounting Office (GAO) study, commissioned by Sen. Edward Kennedy (D-MA) and inspired, in part, by ACCC's focus on the off-label issue, yielded similar data. The report suggested that as many as 106,000 new cancer patients in 1 of 4 major disease sites were not receiving their physicians' treatment of choice because of reimbursement problems.

As we traveled throughout the country over the past 18 months, holding regional meetings on reimbursement and other issues, we polled oncologists in more than 15 states about off-label problems. More than 45 percent of the respondents indicate that denials for off-label use are a problem, albeit an inconsistent one from state to state.

One of the key arguments made by insurers during the early days of the debate was that they did not know who to use as a source for judgments about appropriate treatments other than the FDA. They desired a consistent source, some reference group that was respected, established, and capable of handling the work load. It was in response to this concern that we worked with Congressional aides and selected three nationally recognized drug compendia as a suggested standard. Congress subsequently adopted these three books for use in Medicaid drug reimbursement and, briefly, for Medicare, in the subsequently repealed Catastrophic Coverage Act.

We have written elsewhere about the process by which the three compendia are developed, but it seems clear that they are solid references accepted by Congress, by many state governments, by the Health Insurance Association of America and by many other scientific and governmental sources. To simplify the task of keeping up with the compendia's recommendations on indications, ACCC now publishes a quarterly *Compendia-Based Drug Bulletin,* which is distributed to more than 18,000 cancer care providers and insurers in the United States.

How Well Do Compendia Document Off-Label Use?

The question remains: how much off-label use is recognized by the compendia? To answer this question, we utilized ELM Services, Inc.'s proprietary cancer database to study the off-label use of 10 major chemotherapeutic agents.

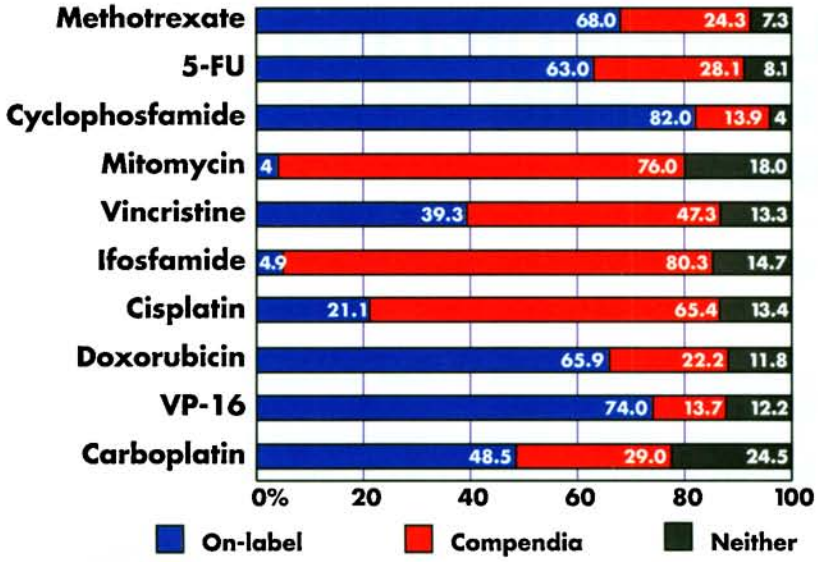
The study gathered information from 77 U.S. hospitals that provide ELM with complete information on all of the cancer patients receiving chemotherapy. A total of 8,743 consecutive patients who received at least 1 of 10 designated chemotherapeutic agents from 1989 to 1991 were selected for study. Information was sorted by site, and percentages were generated for each site-specific use. Each category of use was compared with indications on the label and in the compendia.

Exhibit 1 on the next page illustrates the percentage of medical judgment and use reflected by the three compendia for these 10 agents. When the label and the compendia are combined, the percentage of "unsupported use," or use that might be supported by additional peer-reviewed literature or local technology and science assessment committees, falls to a low of 7.3 percent (methotrexate) and is no more than 24.5 percent (carboplatin).

If one relies only on the FDA label, less than five percent of the current uses of some drugs, most notably ifosfamide and mitomycin, is covered. This puts these agents in high jeopardy for arbitrary insurance coverage policies that confine payment to only the labeled uses. While such a practice does not often occur, for a short

Lee E. Mortenson, D.P.A., *is Executive Director, ACCC, and President, ELM Services, Inc.*

Exhibit 1. Percent of Medical Judgment/Use Reflected by Label and Compendia



time, one Medicare carrier did determine that everything off the label was experimental until members of the state legislature made the carrier recant that policy. Perhaps as important, everything that is off label is subject to payment delays, because insurers force physicians to document their use.

The compendia serve as a major stopgap between the label and incessant case management. Exhibit 2 further illustrates their ability to document a significant proportion of off-label use. From these data, it is apparent that somewhere between 50 percent and 85 percent of off-label use is recognized by the compendia. While other means may be available to explain the remaining percentage of off-label use, the adoption of the three compendia as a standard should eliminate a significant proportion of the unnecessary paperwork that is currently delaying payment and exhausting precious medical oncology resources.

How pervasive is off-label use today? Exhibit 3 indicates that 54.1 percent of all of the chemotherapy patients in the study received one or more of 10 major chemotherapeutic drugs off label. If one examined all of the antineoplastic agents that these patients received during the course of their treatment regimens, it would not be difficult to document that perhaps as many as 90 percent of all cancer patients are receiving at least one of their drugs off label.

Exhibit 2. Percentage of Off-label Use Indicated by Compendia

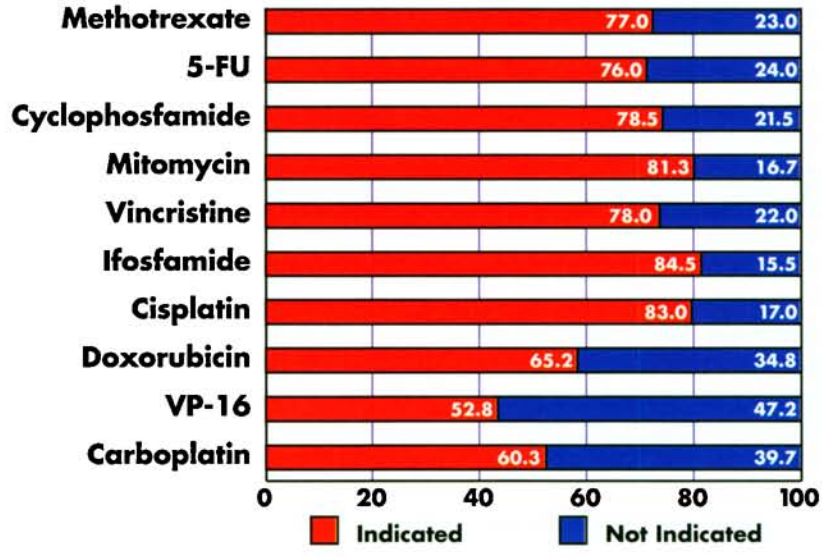
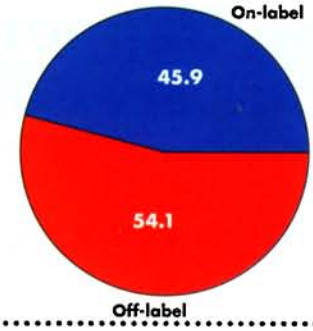


Exhibit 3. Patients Receiving One or More Major Drugs Off-label



While many sources have adopted the three compendia as a standard, some of the less competitive insurers who are losing market share are attempting to cut costs by refusing to accept the compendia as a standard and by insisting on case management instead. While insurers have often stated that case management is more costly for their plans, it presents an additional hassle factor and increased costs for oncologists who wish to order agents that are new or not standard or not on the label. As a result, one patient's case management may cost more, but the savings will come from all of the additional patients who oncologists hesitate to fight about. It is a nihilistic cost containment strategy.

Certainly we have witnessed one case-book example of the difference labeling can make in the selection of closely comparable drugs in the battle for market share that is occurring between GCSF and GMCSF. A separate survey of hospital use of these two products was conducted by ELM during the past six months. It indicates overwhelming market dominance by GCSF, the product with the broadest labeling. Survey data from ACCC regional meetings indicate that many oncologists believe the two agents are likely to be effective for most of the same uses, but a significant number are hesitant to use GMCSF because of its narrower labeling and the possibility of subsequent reimbursement denials or delays.

These data indicate the size and potential of the off-label problem. They also indicate the value of the compendia in explaining off-label use. Without a doubt, universal adoption of the compendia as a standard will significantly improve both reimbursement for and patient access to appropriate therapies.