Coverage Policy and Use Beyond the FDA-Approved Label

The issue of insurance coverage for the use of drugs and biologics in cancer care for indications beyond Food and Drug Administration (FDA)-approved labeling is one that has been important to the oncology community since interferon was approved for use in hairy cell leukemia in the late 1980s. Today, the research pipelines of the pharmaceutical and biotech industries are replete with innovative and promising agents to treat solid tumors and hematologic malignancies and for supportive care.

The approval and use of these important new therapies usually come with significant price tags in addition to improved therapeutic indices. Recent articles focus on the added cost created by integrating new agents into existing regimens. Thus, introducing new agents into practice coincides with a resurgent and growing concern about the rate that health care expenditures rise in both the public and private sectors, with particular emphasis in the Medicare program.

This commentary provides historical perspective on the issue of payment for uses beyond the FDA label, describes the issue within the context of today’s environment, discusses related issues that may impact access to improved therapies, and describes the NCCN’s response.

The introduction of interferon in the 1980s focused the attention of payors on an already common practice in oncology. This biologic agent was approved for the aforementioned “orphan” indication but was quickly also studied and used in patients with a range of solid tumor types and hematologic malignancies. Insurers morphing into managed care companies began to assert their new orientation and managed to begin denying coverage for many of the new uses. Ultimately, this scenario resulted in the passage of legislation, now in 40 states, that requires insurance plans to cover uses beyond those approved by the FDA if such indications were recognized by 3 compendia: the USP-DI, The American Hospital Formulary Service Compendium, and AMA Drug Evaluations. In 1993 an amendment to the federal Omnibus Budget Reconciliation Act required the then Health Care Financing Administration to develop a regulation that was consistent with state laws regarding the use of drug compendia in both national and locoregional coverage policies and decisions. Notwithstanding these state and federal laws, use beyond FDA-approved labeling remained a major and contentious issue during much of the 1990s.

The level of contention subsided as health care plan managers decided that the adverse public reaction to treatment denials, concern of major employer-customers, and large punitive legal judgments warranted a more hands-off approach and less intrusion into the decisions made by physicians and patients. However, the issue was raised anew in January 2004 in The New York Times when a senior official at the Centers for Medicare and Medicaid Services (CMS) indicated that CMS was considering becoming more limiting in its coverage for use beyond FDA-approved labeling. Clearly, CMS, charged with the huge task of implementing the Medicare Drug Benefit, was facing intense pressure to manage the costs of that benefit. After negative reaction, the trial balloon floated in The New York Times article was purposefully deflated. However, although CMS as a national decision maker deferred, one or two Medicare in-
Use beyond FDA-approved labeling in the rapidly evolving area of cancer care is a common and important practice, with estimates of the extent of such use usually in the 50% range. The timelines for publishing study results, requirements and timelines for seeking FDA approval for supplemental indications, timelines for establishing coverage policies, and impact of these events on therapeutic decisions are beyond the scope of this article. Rather, this article discusses the compendia legally recognized as authoritative sources for establishing coverage policy, including their shortcomings, and laws that can impact access to care for cancer patients.

Firstly, the mandated compendia have dwindled. The only compendium (AMA Drug Evaluations) developed directly by the practicing medical community no longer exists, and The USP-DI name will only exist through 2007. More importantly, existing compendia can be deficient in keeping pace not only with the rate of scientific publications but even with FDA approvals. As indicated in the January 28, 2005, CMS national coverage decision on agents used to treat colorectal cancers, bevacizumab (approved 11 months earlier in February 2004) was not even listed in The USP-DI. Although the compendium is working to address this issue, this fact points out the need to ensure that legally recognized compendia communicate recommendations that reflect up-to-date scientific results and expert judgment.

Recognizing the challenges presented to clinical professionals in oncology, and building on the wide use of the NCCN Clinical Practice Guidelines in Oncology in setting coverage policy, the NCCN has launched the Drugs and Biologics Compendium. This compendium seeks to:

- Represent and communicate the recommendations of expert clinicians on the appropriate uses of drugs and biologics in cancer care to constituencies, including payors, who make decisions that impact patient access;
- Communicate the same recommendations to decision-makers in oncology practice;
- Achieve legal and regulatory recognition (federal and state) and status as a standard for the appropriate use of drugs and biologics in cancer care.

In doing so, it is important to note that:

- Recommendations in the NCCN Drugs and Biologics Compendium are derived directly from the NCCN Clinical Practice Guidelines in Oncology, widely used and recognized as the standard for clinical policy in oncology;
- All categories of NCCN recommendations are deemed to represent appropriate care for patients;
- The NCCN guidelines and the NCCN compendium maintain the most rapid updating in the health care industry;
- The NCCN compendium focuses on the broad continuum of uses of drugs and biologics in cancer care;
- The NCCN compendium is developed by thought leaders from the oncology community based on their evaluation and analysis of available scientific evidence integrated with expert clinical judgment.
In summary, as advances in pharmacotherapy and biologics therapy accelerate and proliferate, coverage policies and decisions that impact the availability of appropriate treatment to patients with serious and life-threatening illnesses must be based on timely, scientific, and authoritative analyses and recommendations. The oncology community must critically have that voice and make that contribution. The NCCN has committed itself to providing such timely, scientific, and authoritative information freely to all constituencies making these decisions. As the NCCN moves to have the Drugs and Biologics Compendium recognized by CMS as one of several mandated references for coverage determinations, we ask that you add your voice and that of your representative professional organizations to the many individuals and organizations working with the NCCN and CMS to ensure that coverage policies enable access to appropriate treatment for the patients whom we serve. (For more information or to view the Drugs and Biologics Compendium, please go to www.nccn.org.)

References