ASCO's Clinical Practice Committee

Following the Pipeline

Peter Paul Yu, MD

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The three most important clinical practice roles we assume are coordinating a multidisciplinary cancer plan, providing long term and end-of-life care, and implementing a therapeutic plan. Arguably, implementing a therapeutic plan commands the most commitment from us and is the most under threat. The Clinical Practice Committee

(CPC) addresses access to new drugs, costs to the health system, and fair reimbursement for supplying these drugs to patients by working with the relevant government agencies—the US Food and Drug Administration (FDA), the National Cancer Institute (NCI), and Centers for Medicare and Medicaid Services (CMS). These are the critical areas I see the CPC watching during the next year.

FDA

In the 1980s, new drug development began to shift toward research targeted against newly discovered signal transduction and apoptotic pathways. However, the expected cascade of promising new drugs has not yet materialized. Over the last 5 years, the number of applications to the FDA for new drugs representing novel chemical structures, have fallen, despite the more than doubling of research funding by both the National Institutes of Health and private industry.¹ Further, the responses from targeted therapies may be applicable only to patients with specific genetic mutations, illustrated by gefitinib and non-small-cell lung cancer. In response, the FDA has initiated the Critical Path project to streamline new product development. Their aim is to accelerate drug development and to determine which drugs are effective and less toxic early in the development process. Knowledge of specific signal transduction pathways should afford drug developers opportunities to measure biologic effect on these pathways as a surrogate for clinical benefit. Understanding the complexities of interacting signal transduction pathways should inform design of efficacy studies of new and potentially more effective drug combinations.

NCI Clinical Trials Operation

The NCI has accepted the Clinical Trials Working Group recommendations to prioritize future trials, standardize elements of the research process (such as case report forms

and contracting), build an NCI-recognized credentialing system for researchers and sites, and create a national clinical trials information technology system. The unintended consequences of some proposed changes—increasing accrual targets, required information technology systems, and mandatory accreditation—may place a greater financial burden on oncology practices and threaten clinical trial participation.

CMS and Medicare

Nowhere has the impact of change been more evident to oncologists than with CMS. While the National Institutes of Health and FDA expand and refine the therapeutic arsenal, CMS struggles to contain the explosive growth in demand for deployment of the medical service arsenal. Although provisions of the Medicare Modernization Act affecting payments for drugs have received the greatest attention, recent CMS decisions regarding off-label policies are also important.

FDA drug indications approval and CMS coverage policy are not aligned. More than half the uses of anticancer chemotherapy drugs are for indications other than those approved by the FDA—so called “off-label” use. An existing avenue for Medicare coverage of off-label use is acceptance and listing in one of two drug compendia specified in statute, the United States Pharmacopeia Drug Information (USP-DI) and the American Hospital Formulary System. Currently, the USP-DI, published by Thomson Micromedex (Greenwood Village, Colorado), is the primary source for oncology drugs. CMS is reviewing addition of the National Comprehensive Cancer Network drug compendium to its recognized list. Medicare Part B carriers in each state can also make off-label coverage decisions for their local jurisdictions. Though carriers are required to consider articles published in peer-reviewed medical journals, the secretary of Health and Human Services has only recognized nine journals as sufficiently rigorous and authoritative. As newer journals have become important sources of trial results, the list has not changed. ASCO is petitioning the secretary to recognize additional journals. Furthermore, contrary to statute and regulation, some carriers have indicated a preference to rely solely on the compendia listings. ASCO has been active in reminding individual carriers of the requirement to consult published literature in making coverage decisions. ASCO also hosts an annual meeting for oncologists serving on the state Carrier Advisory Committees to identify carrier-specific problems and solutions.

CMS has begun to consider how the results of treatments performed on research populations translate to the general Medicare beneficiary population. Because general population data are usually unavailable, CMS has developed a policy of Coverage with Evidence Development. It is intended to allow CMS to acquire data on the efficacy and value of cancer diagnostics and therapeutics. These services will be provisionally reimbursed in exchange for data acquisition, pending a later decision on coverage.

The least costly alternative rule allows CMS to limit reimbursement of drugs to that of a less costly alternative that CMS considers to have equivalent efficacy. While the rule is not a new one, these judgments as to what constitutes therapeutic efficacy will receive close attention by the CPC.

Reference

1. US Department of Health and Human Services, Food and Drug Administration: Innovation stagnation: Challenge and opportunity on the critical path to new medical products. <http://www.fda.gov/oc/initiatives/criticalpath/whitepaper.html>

By the People, for the People

Fair reimbursement for drugs and services is impacted by all of these policy issues. The importance of an organization such as ASCO, which can recognize the complexity of the problem and work with all parties to achieve compromises and solutions to difficult issues, is paramount. The CPC represents the concerns of practicing oncologists in these discussions. Our efforts next year will impact these policies, and we ask for your help and support. My future columns will detail our progress, and suggest ways you can be involved.

Peter Paul Yu, MD, is a medical oncologist with Camino Medical Group, a division of the Palo Alto Medical Foundation in Sunnyvale, California. He is currently chair of ASCO's Clinical Practice Committee.