

National Coalition for Cancer Survivorship Industry Roundtable: Why Reimbursement Matters



Meeting Summary

Prepared by The Health Strategies Consultancy, LLC



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I. Summary

A. Purpose

Reimbursement is of critical importance to oncology patients because the way in which payers reimburse creates incentives – and disincentives – for providers to use state-of-the-art technology for cancer care. Put simply, physicians, patients and policy makers respond to financial incentives imposed through payment and billing rules. The desire of clinicians to use therapies is often defined by payment environment. Therefore, it is critical that reimbursement mechanisms are designed to ensure that there are incentives to use good technology and to avoid using dangerous or inferior technology.

Due to reimbursement's potentially significant impact on patient access to care, it is an important area for patients and advocacy groups to understand. Reimbursement is equally important to industry, as it can have a substantial impact on product sales, overall revenues, and planning for the development of new products. Thus, both advocacy groups and industry have a shared interest in ensuring that reimbursement creates incentives for providing the most appropriate care to cancer patients and for developing innovative cancer therapies.

Given the importance of reimbursement to both industry and advocacy groups, the National Coalition for Cancer Survivorship (NCCS) convened an Industry Roundtable on March 3-4, 2003 in Washington, DC, which focused on the range of reimbursement issues plaguing drug development and the delivery of quality cancer care.

Specific goals of the Roundtable included:

- Providing a better understanding of the future challenges of reimbursing cancer care in a variety of settings.
- Discussing how trends could possibly affect the patient, and the potential impact on future advocacy agendas.
- Educating participants regarding the FDA processes, legislative initiatives influencing the reimbursement of cancer care and the potential impact on the patient.

B. Faculty and Agenda

The meeting was facilitated by Dan Mendelson, President of The Health Strategies Consultancy LLC. Additional faculty included Gregory J. Glover, MD, Ropes & Gray, who spoke on intellectual property and the FDA approval processes; Jenifer Levinson, The Health Strategies Consultancy, who spoke on reimbursement for oncology services; Richard Pazdur, Director of Oncology Drug Products, Center for Drug Evaluation and Research, Food and Drug Administration, who spoke on oncology-specific FDA initiatives; and Samuel Turner, Ropes & Gray, who spoke on legislative initiatives pertaining to oncology reimbursement.

The first day of the March 2003 Roundtable was attended by approximately 40 individuals representing the pharmaceutical and biotechnology industry and 19 oncology advocacy groups. On the second day, attendance was limited to industry representatives.

The agenda on day one of the Roundtable was focused on providing expert instruction in the areas of oncology reimbursement and regulation. Following introductions, the agenda included a session on “Reimbursement 101”, a summary of legislative issues, an overview of the FDA regulatory process, and perspectives from the FDA. The day’s agenda concluded with a discussion of the future of cancer care. The agenda for day two was more open, beginning with an overview of key points from day one, and concluding with a facilitated discussion of how industry and cancer advocacy groups can work together to address key reimbursement issues. A copy of the full agenda is included as Attachment 1.

C. Conclusions

Access to medical technology is a crucial issue for both industry and advocacy groups and thus is an area ripe for collaboration. In this context, it is possible to identify mutual priorities across the reimbursement spectrum, including coverage, coding and payment:

- **Coverage:** When payers act to limit coverage to cancer treatments, patients may not have access. A current example is Medicare’s national coverage review of oxaliplatin, a colorectal cancer treatment. CMS is evaluating the drug to determine whether Medicare should cover it for its FDA-approved indication.
- **Coding:** Coding is important in that it enables providers to fill out claims and get paid for services. Delays in assignment of appropriate coding can mean that providers do not get paid for a new service, thereby deterring use. Currently, it takes more than a year to obtain appropriate coding for new chemotherapies.
- **Payment:** When providers are paid inadequately for services, they can’t afford to provide them. Both patients and industry suffer when payment is inadequate to cover the cost of services. Technologies that are underpaid in the long-term may be withdrawn from the market, even if they offer clinical benefits to patients.

Education on reimbursement issues is essential to ensure that advocacy group and industry leadership is fully aware of the importance of reimbursement. Critical oncology reimbursement issues exist today, and these issues will continue to grow in importance as the population ages, cost pressures increase, and costly new technologies are introduced in the market.

In the future, reimbursement decisions will determine crucial factors related to patient access to cancer care, including patient cost burden / ability to pay, physician incentives to use technology, and financial return for research and development. The course is being set now for the future, in current deliberations on the shape of public programs (Medicare, Medicaid) and trends in managed care. Patients and industry must educate themselves and be engaged on these issues now, in order to ensure that both groups’ fundamental objectives in terms of access to cancer treatments are met.

II. Overview of Oncology Reimbursement Issues

A. Why Reimbursement is Important

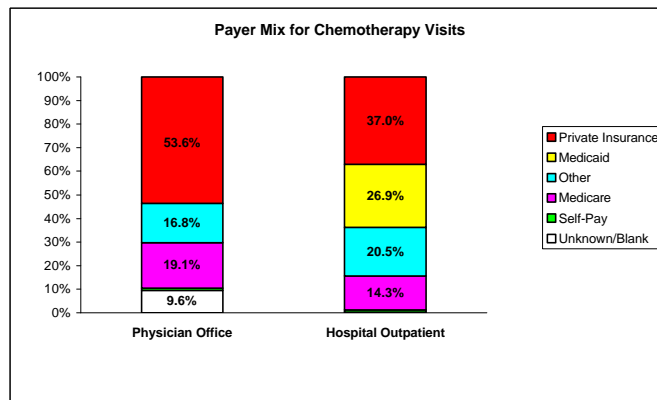
Reimbursement is a critical factor affecting cancer care because different payment systems can create incentives – and disincentives – for providers to use state-of-the-art technology for cancer care. Put simply, physicians, patients and policy makers respond to financial incentives. The desire of clinicians to use therapies is often defined by payment environment. Therefore, it is critical that reimbursement mechanisms are designed to ensure that there are incentives to promote the use of good technology and discourage the use of dangerous or inferior technology.

Because of reimbursement’s potentially significant impact on patient access to care, it is an important area for patients and advocacy groups to understand. Reimbursement is equally important to industry, as it can have a substantial impact on product sales. Thus, both advocacy groups and industry have a shared interest in ensuring that reimbursement creates incentives for providing the most appropriate care to cancer patients.

B. Basics of Coverage, Coding and Payment

Reimbursement for oncology services in the US is highly fragmented. A variety of payers finance health care in the US, including public payers (Medicare, Medicaid, the VA, and the Department of Defense) and commercial payers (managed care and indemnity plans). Although many payers contribute to oncology care, the majority of oncology services are paid by private insurers, Medicare, and Medicaid (Exhibit 1).

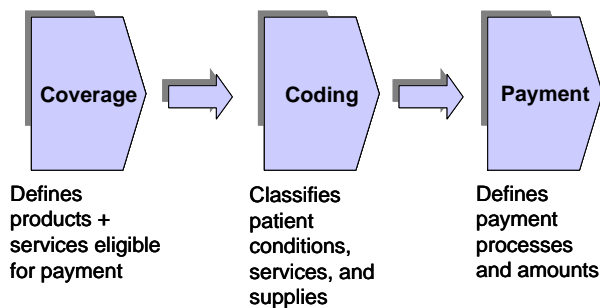
Exhibit 1: US Payer Mix for Chemotherapy



Source: Health Strategies Analysis of 2000 NAMCS and NHAMCS.

Reimbursement varies by payer and by the site of service in which care is delivered. However, the three primary components of reimbursement, coverage, coding, and payment, are consistent across all payers and payment systems (Exhibit 2). Each of these components is described in greater detail below.

Exhibit 2: Key Components of the Reimbursement Process



Coverage

Coverage describes products and services eligible for payment. Coverage is typically outlined in insurance contracts, and usually includes services that are safe and effective, not experimental or investigational, and appropriate to patient or treatment setting. Payers institute coverage criteria in order to ensure appropriate utilization. In general, FDA approval is required for payers to cover a service. However, FDA approval is not usually sufficient for coverage. Payers typically look for evidence of added value relative to existing treatment options. Products and services providing added value might include those offering: 1) substantial improvements in health outcomes otherwise obtained by currently covered items or services, 2) access to a different clinical modality, or 3) substitution for an existing item or service at a lower cost. Specific coverage issues for the three major payers for oncology services, Medicare, Medicaid, and commercial payers, are described below.

- **Medicare:** There are two basic thresholds for Medicare coverage: the legal/statutory parameters of the Medicare program and whether a service is “reasonable and necessary”. In terms of legal parameters, Medicare can only cover items that are FDA-approved (with a few exceptions) and fall into a statutory benefit category. Notably, these benefit categories do not include screening and prevention or self-administered drugs/biologicals. However, there are some important exceptions in oncology. Medicare covers several types of cancer screening (mammography, PAP smears, prostate cancer screening, and colorectal cancer screening), and covers certain oral anti-cancer drugs (those with an intravenous equivalent) and oral anti-emetics. In addition, Medicare has specific regulations regarding the circumstances in which off-label uses of oncology drugs must be covered.

If a product fits into a statutory benefit category, then Medicare makes coverage decisions in one of two ways: locally or nationally. The majority of coverage decisions are made locally, by the local contractors who process Medicare claims. For high-cost/high-profile items, there is a national coverage process, which includes formal timeframes for review. Any interested party can request a national coverage decision, and the Agency can also institute such decisions independently.

- **Medicaid:** Medicaid is a joint federal/state program. There are broad federal guidelines that states must follow, including mandatory services (physician services, hospital inpatient and outpatient services, well-child care, family planning) and optional services (drug benefits, PT, OT, speech therapy, dental services, vision care). States establish eligibility standards, benefits packages, payment rates, and program administration.

Most coverage decisions for new technologies are made at the state level. However, federal statute guides the provision of outpatient drug benefits. With a few exceptions for “lifestyle” drugs (e.g., weight loss drugs, fertility drugs, etc.), if a manufacturer signs a rebate agreement with the state, the state Medicaid program must cover any FDA-approved drug from the manufacturer, at least for labeled indications (as with Medicare, Medicaid must cover off-label indications of cancer drugs in certain circumstances). To control drug costs, states use prior authorization for high cost drugs (including some cancer drugs) and preferred drug lists (PDLs), which operate like formularies and allow states to obtain additional manufacturer discounts in exchange for “preferred” status.

- **Commercial Payers:** Most commercial payers classify services into a medical or a pharmacy benefit. Historically, most oncology drugs administered in the physician office or clinic setting were classified as a medical benefit, and thus were not subject to formularies, prior authorization, and other common utilization control mechanisms. Currently, some managed care plans are moving oncology drugs into the pharmacy benefit in order to better control costs for these drugs. Regardless of the benefit category, commercial payers typically make coverage decisions based on product safety, efficacy, and cost-effectiveness. As with Medicare, most commercial payers cover off-label uses of cancer drugs if there is sufficient evidence showing effectiveness for the off-label indication.

In summary, FDA approval is necessary for coverage, but is usually not sufficient. Although coverage is the first step in providing access to cancer treatments, coverage does not ensure that a medical service or technology will be adequately paid. Finally, Medicare is a key player in coverage decisions for cancer care. Commercial plans and Medicaid often look to Medicare for guidance on new technology coverage decisions.

Coding

Coding is important because it enables providers to submit claims to payers. There are five national coding systems relevant to oncology services, and each coding system relates to payment in unique ways, depending on the setting of care and the payer (Exhibit 3). Coding systems are updated annually, and timing of coding updates plays an important role in providers’ ability to obtain adequate reimbursement for new cancer products and services.

Exhibit 3: Coding Systems

Coding System	Maintained By	Uses/Settings
ICD-9-CM International Classification of Diseases, Clinical Modification, 9 th edition	<ul style="list-style-type: none"> • Procedures: CMS • Diagnoses (Dx): NCHS 	<ul style="list-style-type: none"> • Hospital inpatient (DRG) • Outpatient settings (Dx) (medical necessity)
CPT-4 (HCPCS Level I) Current Procedural Terminology, Version 4	• American Medical Association	<ul style="list-style-type: none"> • Physician • Laboratory • Hospital outpatient (APC)
HCPCS (Levels II, III) Healthcare Common Procedure Coding System	• CMS	<ul style="list-style-type: none"> • Drugs and biologicals • DMEPOS • Services not in CPT • HOPPS pass-through codes
Revenue Codes	• NUBC/AHA	• Hospital inpatient/outpatient
National Drug Codes	• HHS (FDA)/Drug manufacturers	• Drugs and biologics/pharmacy

Payment

Payment for cancer drugs and other services varies by payment mechanism. Incentives related to drug use also depend upon the payment mechanism. Currently, most payers pay separately for injectable oncology drugs when they are administered in the physician’s office. These payments currently tend to be based on the drug’s average wholesale price (AWP), which is a price established by the drug’s manufacturer. Medicare pays 95% of AWP for drugs administered in the physician office; commercial payers negotiate discounts individually with manufacturers. Medicaid payment for physician-administered drugs varies widely by state – some states pay more than Medicare, some pay the same as Medicare, and other states pay based on the physician’s acquisition cost for the drug. Discounts are also negotiated for drugs that patients obtain in the outpatient pharmacy setting. Under Medicaid, states receive a mandatory rebate from manufacturers for these drugs; commercial payers negotiate price with drug manufacturers.

Outside of the physician office setting, payment mechanisms also vary by payer. In the hospital outpatient setting, the other major setting in which chemotherapy is administered, Medicare recently implemented a system whereby drugs are treated in one of three ways: (1) new drugs (“pass-through drugs”) are paid at 95% of AWP; (2) higher cost drugs (defined at >\$150 per dose in 2003) are paid based on hospital costs for purchasing the drugs, where hospital costs are based on prior year Medicare hospital claims; and no separate payment is made for lower-cost drugs. Commercial payers still tend to pay hospital outpatient departments for drugs and other services based on the charges reported on claims.

C. Current Legislative Issues

The Environment

There are a number of challenges confronting Congress and the Administration relative to health care legislation. Most importantly, the promise of a Medicare prescription drug benefit puts pressure on both parties to produce results before the 2004 elections (see below), but budget constraints and philosophical differences make realizing this goal difficult. Meanwhile,

Medicare coverage has become increasingly costly with the introduction of expensive new therapies and greater levels of utilization. Furthermore, the rising wave of “baby boomers” eligible for Medicare, along with their greater longevity poses a real threat to the solvency of the Medicare program.

From a patient care perspective, there a number of weaknesses of the current Medicare program that need to be addressed, including the lack of an outpatient drug benefit, limited coverage of prevention services, restricted diagnostic screening benefits, even where proven effective, and uncertain funding for the future. Existing Medicare payment systems are also flawed in terms of accommodating new and important medical technologies.

Similar constraints exist in state health care programs. The State Children’s Health Insurance Program (SCHIP) program has expanded health insurance coverage by an additional 500,000 children, but the economic downturn has created massive shortfalls in most states, with 45 million now dependent on Medicaid. Two-thirds of the states are embarking on a second round of cuts halfway through their fiscal years. Many states are taking drastic measures to reduce costs: 40 states are planning to implement pharmacy controls, 29 states are planning to reduce or freeze provider payments, 15 states will reduce Medicaid benefits, and 18 states will restrict Medicaid eligibility. To address state budget concerns relative to Medicaid, the Bush Administration is proposing fixed payments to states over ten years in exchange for state flexibility in administering their Medicaid programs.

Further complicating the scenario is a heightened focus on health needs arising from the anti-terror effort, including “bio-terrorism”, a catchall category that covers new defenses against toxins as well as traditional vaccines. For example, the Bush budget proposal urges an expansion of the controversial “Vaccines for Children” program, funding larger vaccine stockpiles and increasing funding for vaccine purchases. Prevention is also on the agenda, and the Bush budget proposal augments the Centers for Disease Control and Prevention (CDC) budget by dedicating \$100 million to prevention of chronic diseases such as diabetes, obesity and asthma. Other overarching themes include cost containment and private/market-driven approaches to health financing and delivery.

Medicare Prescription Drug Benefit

A major area of legislative debate continues to be the addition of a Medicare prescription drug benefit. The most current Bush Administration proposal offers a choice of a drug benefit under a managed care plan (similar to Medicare+Choice) or an “enhanced fee-for-service” plan with a drug benefit run by the private sector, operating like a preferred-provider organization (PPO). Compromise options also being discussed include providing older retirees with a drug benefit while staying in traditional Medicare, and entering younger retirees (55 and under) into a system similar to the Federal Employees Health Benefit Plan (FEHBP) as they retire, with a defined government contribution and a choice of plans. Other proposals offer more generous benefits in traditional fee-for-service Medicare, but most proposals trade-off between high copayments and premiums, gaps in coverage, and high costs for implementation.

Based on this environment, there appear to be two possible scenarios. One scenario is continued gridlock, as in the last Congress, with prescription drug coverage passing the Republican House

and failing in the Senate. Republicans will blame Senate Democrats and hope for a replay of the 2002 mid-term elections. Alternatively, we could see a scenario similar to the 1987-88 passage of the Medicare Catastrophic Coverage Act (MCCA). In this case, gridlock would continue until the election nears, with a last minute drive to compromise and enact some type of compromise legislation. The fate could then be similar to that of the MCCA. After passage of the MCCA, Congress was besieged by seniors concerned about too-high premiums (and general disappointment with the benefit), and the benefit was repealed the next year.

Oncology-Specific Legislation

Congress is considering several important pieces of oncology legislation. The two primary pieces of legislation of critical importance to the oncology community include Medicare coverage of oral anti-cancer drugs and reform of average wholesale price (AWP).

- **Oral Anti-Cancer Drugs:** Legislation, entitled the “Access to Cancer Therapies Act”, was recently introduced in the 108th Congress by House Republican Conference Chair Deborah Pryce and Representatives Lois Capps, Steve Israel, and Sue Myrick expands Medicare coverage to all oral anti-cancer therapies. The Access to Cancer Therapies Act was supported by 328 Representatives and 57 Senators in the 107th Congress.
- **AWP Reform:** Congress continues to focus on reducing Medicare payments for covered drugs. Because oncology drugs represent a substantial portion of the drugs currently covered by Medicare, the oncology community will be disproportionately impacted by any changes in the current 95% AWP payment formula. A number of proposals have been discussed in Congress, including competitive bidding (similar to managed care contracting for drugs), and reimbursement based on a newly defined price term, the “average sales price” (ASP). The ASP method would ensure that the Medicare program receives a competitive price for drugs relative to other preferred customers, including managed care plans.

Of particular concern for the oncology community is that currently, drug reimbursements offset underpayments by Medicare for other oncology services. Therefore, physician and patient oncology advocacy groups are trying to ensure that payments for oncology services such as chemotherapy administration are increased in proportion to any reductions in drug payments.

Each of these issues will be addressed in context of the environmental challenges described above, and could be handled independently or incorporated into broader Medicare reform plans.

D. Summary of Key Trends

As described above, there are a number of critical reimbursement issues facing the oncology community today. A summary of key trends is provided below.

- **Medicare coverage:** Medicare has begun to initiate national coverage decision reviews of oncology drugs for their FDA-approved indications. In the last six months, CMS has internally initiated national coverage reviews of two oncology treatments for their FDA-approved indications, ibritumomab tiuxetan (Zevalin) for Non-Hodgkin's Lymphoma and oxaliplatin (Eloxatin) for colorectal cancer. Previously, Medicare typically covered FDA-approved indications of cancer drugs without formal review. Another trend in Medicare coverage is an increased focus on collaboration with the FDA in terms of making coverage decisions.
- **Medicare payment:** Drug payments in the physician office and hospital outpatient settings continue to be controversial. Legislative proposals to eliminate the 95% AWP-based payment for Medicare covered drugs delivered in the physician office were discussed above. In addition, CMS has promised to revise AWP-based payments independently if Congress does not act. Although CMS is mandated by law to pay at 95% AWP, the Agency could “redefine” AWP in order to reduce payments. There is strong sentiment from the pharmaceutical industry that drugs are paid inadequately under Medicare’s hospital outpatient prospective payment system (OPPS). Although there has not been general consensus among the industry on an appropriate alternative, various companies and industry groups are engaged in conversations with CMS and Congress about increasing drug payments under this payment system.
- **Commercial payment:** Among commercial payers, an important trend is increased use of patient cost-sharing requirements. In particular, as managed care plans attempt to reduce their costs for high-cost injectable products (including cancer chemotherapy), they are shifting these drugs to the pharmacy benefit, where they are subject to copayments. In some cases, managed care plans are also creating third- and fourth-tiers in their formularies for high-cost drugs, with very high patient copayments. Other utilization control techniques that are being applied to oncology drugs include prior authorization and step therapy.
- **Medicaid:** As described above, states are focused on decreasing their Medicaid costs. Because prescription drugs form a large component of these costs, many states are looking at new ways to manage these costs. Many states have implemented or are in process of implementing preferred drug lists (PDLs), prior authorization programs, and other restrictions on outpatient drugs. We expect that this trend will carry over to high-cost injectable drugs, potentially including chemotherapy, in the future.

E. Implications for the Future

Environmental issues and assumptions

The US health care system will continue to face cost pressures while attempting to integrate exciting new health care advances. Forecasting requires certain assumptions about the health care environment in light of these pressures. Our sector-specific projections are based on the following assumptions regarding the future environment:

- **Health care costs will rise across sectors:** The respite from a rise in hospital costs appears over, and pharmaceutical spending continues to rise more rapidly than other sectors. Negative scrutiny and blame on pharmaceutical sector for continued rise in costs continues. Manufacturers may feel the need to increase price to meet earnings. Activity encourages development of more crude formularies over time.
- **Change in insurance penetration/uninsured:** A continued soft economy and corporate weakness fuels an increase in the uninsured, and efforts to expand Medicaid and SCHIP do not overcome the loss of private insurance. In fact, with state budgets in crisis, millions are cut from the Medicaid roles. Lack of insurance expansion results in more exposure of patients to **pharmaceutical** costs, and rising costs and loss of **insurance** increases attention to healthcare as a political issue.
- **Tolerance for cost sharing:** Insured individuals will continue to expect that expensive medications are covered, and employers and insurers will continue to try to shift more responsibility to patients. Unions / patient groups are starting to push back in the current environment, and payers will respond by linking co-pays to tighter formularies and policies related to “lifestyle”. The “fourth tier” (high copay) formulary concept will continue to spread but not as rapidly as insurers would like.
- **Strength of managed care environment:** Expect some growth in more tightly managed care with economic weakness. Managed care will continue to increase leverage in pharmaceutical negotiations. Aggressive attempts to constrain formularies and shift biotech costs to patients are expected.
- **Medicare policy decisions:** Comprehensive reform remains elusive, but a limited drug benefit is possible in the coming years via drug discount cards, a dual eligible policy, and/or a low income / Medicaid solution. (Note: all major “comprehensive” drug benefit proposals currently cover less than 35% of drug costs.) Injectables and drugs administered in the physician office retain relatively generous co-payment rates.
- **Oncology-specific Medicare decisions:** Oral cancer drugs are included in the Medicare drug benefit. A formulary is also part of the drug benefit, and Medicare has broad discretion to create and enforce formularies by therapeutic category. Medicare begins to use specialty pharmacy to create competition / manage costs.
- **Medicare payment legislation:** Improvements in coverage and payment for screening / prevention are passed. No other major changes in payment systems are implemented in the near future, although there continues to be discussion of the role of new technology payments and bundling. Although OPSS failures are highlighted, there is a continued push towards bundling as a way to save money.
- Sector-specific projections are outlined below.

Sector-Specific Forecasts

Medicare

Under the environmental assumptions outlined above, likely short-term changes in Medicare might include the following:

- AWP reform, combined with some increases in payments to physicians for drug administration, and increased use of other mechanisms to reduce Medicare payments for covered drugs.
- Lengthier/delimited coverage determination process for new treatments and diagnostics.
- Broader coverage for injectable drugs that can be self-administered.

Other potential near-term changes in Medicare include coverage of oral cancer drugs, expanded coverage of preventive benefits, disease management, and increased Medicare managed care options.

Medicaid

Current trends in Medicaid will continue to expand, including introduction of PDLs and expansion of PDLs and other cost containment mechanisms to injectable drugs, including cancer treatments. In addition, states will cut back their enrollment in Medicaid, leaving millions without coverage.

Commercial / Managed Care

In the commercial payer/managed care sector, plans will continue aggressive application of formularies, with some expansion to injectables, including cancer drugs. Efforts to increase patient cost sharing will continue. However, there will be some backlash against increased copayments.

Future of Oncology Reimbursement

In the longer term, technology innovations will drive expensive, patient-specific therapies (e.g., gene therapy), and will change the markets for prescription drugs. Meanwhile, demographics will continue to strain public programs, and an increased cost burden will be placed on both patients and insurance companies. To respond to these changes, new models for payment will evolve (e.g., “mortgage” models of paying for high cost care), accompanied by concerns about creating a multi-tiered system of care.

A major risk is that traditional insurance concepts could break down, due to provider non-participation in public programs (and even private insurance plans) due to inadequate payment levels, and as increased genetic information prevents traditional insurance underwriting.

III. Overview of Oncology Regulatory Issues

A. FDA Trends

A number of changes have occurred at the FDA under the Bush Administration. The new FDA Commissioner, Mark McClellan, MD, PhD, is the first-ever political insider to head the agency. He brings a consumer orientation to the Agency, favoring more consumer information (e.g., qualified food labeling), bolstered by physician support for direct-to-consumer (DTC) ads. In addition, the FDA was able to facilitate reauthorization of PDUFA (pharmaceutical user fees) and passage of device user fee regulation, both of which promote shorter FDA review times. There is no sign of support for FDA jurisdiction over tobacco, under the Bush Administration.

There are also several important FDA trends specific to oncology under McClellan. In a recent Wall Street Journal editorial, Commissioner McClellan was given high marks for noting increased review times for drugs for cancer and other life-threatening diseases and proposing action. McClellan has focused on improving communication between FDA and sponsors – making the process more consistent, predictable and transparent. In addition, McClellan would like to incorporate more advice from practicing oncologists in the review of cancer drugs and to encourage collaboration between ASCO and the FDA to develop appropriate end points for cancer clinical trials. Other relevant organizational changes include combining the drug and biologicals centers for most therapeutic products (as opposed to preventive products like vaccines); however, oncology drug reviews will remain separate for the time being.

Comments from Richard Pazdur, Director of Oncology Drug Products, confirm a continued effort to improve FDA approval times for cancer drugs. Pazdur noted that, despite the high toxicity of many cancer drugs, most problems with FDA approvals for cancer drugs are related to efficacy rather than safety. Thus, development of appropriate end points for oncology clinical trials is therefore a priority. Pazdur also encourages more collaboration between the FDA and the National Cancer Institute (NCI) in the drug approval process. In contrast, Pazdur did not express support for increased collaboration with CMS during the drug approval process, although he believes that input from the FDA on clinical issues related to coverage is appropriate.

B. Intellectual Property / Hatch-Waxman Reform

Importance of Intellectual Property

Timing of drug development is of central importance to patients who are seeking new and innovative cancer treatments. Drug development is also expensive, time-consuming, and risky for manufacturers. Typically, development of a new drug costs approximately \$800 million (DiMasi et al., J Health Econ 1991, and Tufts Center for the Study of Drug Development 2001) and takes on average 14 years. Moreover, only 20 of 5,000 compounds screened in preclinical testing and only 1 drug in 5 that reaches human clinical trials is approved by the FDA (DiMasi JA, “New Drug Development in US: 1963-1999.” Clinical Pharmacology & Therapeutics, 2001, May, 69(s)).

Intellectual property protection is important because it offers manufacturers of innovator drugs some guarantees against competition, which helps to mitigate risk and assists manufacturers in recouping drug development costs. Because intellectual property protection provides an incentive for developing innovative new products, it is in the interest of oncology patients to ensure that adequate protections exist. However, this desire must compete with the economic need to bring lower cost, generic products to the market. It is in this context that debate over intellectual property protections for innovator drugs is occurring.

History of Hatch-Waxman

The Hatch-Waxman Act was implemented in 1984; the Act is important because it addresses all aspects of market protection (e.g., patent protection, data protection, and market exclusivity). The purpose of the Act was to balance the need to make low-cost generic drugs available quickly with the desire to maintain incentives for pharmaceutical companies to conduct pioneering research. Major components of Hatch-Waxman include the following:

- Enabled use of patented drug products prior to patent expiration for purposes of obtaining pre-market FDA approval (§271(e) exemption).
- Authorized FDA approval for generic drugs based on “bioequivalence”.
- Provided patent term extensions for innovator drugs to make up for FDA review time.
- Reduced data exclusivity of innovator drugs to 5 years and reduced data exclusivity for additional indications to 3 years.
- Provided 180 days of market exclusivity to first generic drug applicant to challenge innovator patents.

Another key – and controversial – component of Hatch-Waxman is the automatic 30-month stay on FDA approval if the manufacturer of an innovator product brings suit for patent infringement against a generic (ANDA) filing. Multiple suits can result in multiple 30-month stays.

Additional intellectual property protections added subsequent to Hatch-Waxman include pediatric exclusivity, which provides an additional six months of market exclusivity for studies on the pediatric population conducted at the request of the government, and orphan drug exclusivity, which provides 7 years of market exclusivity post-FDA approval for drugs treating a disease/condition affecting less than 200,000 people or for which the sales cannot reasonably be expected to cover the R&D costs.

Proposed Reforms

A number of reforms to the intellectual property protections described above have been proposed. Most of the legislative proposals made during the last and the current Congressional sessions would eliminate or minimize the 30-month stay on FDA approval initiated when the manufacturer of an innovator product files a patent infringement suit against the filer of an ANDA. Other proposals would limit the type and timing of patents that can be listed for innovator products. Meanwhile, the FDA has proposed regulation that would address these

issues in the absence of legislation. The FDA proposal would limit the types of patents that could be challenged under the 30-month stay and would allow only one 30-month stay per ANDA.

IV. Where Joint Priorities Exist Between Patients and Industry

A. Interaction Between the Regulatory and Reimbursement Processes

Given the critical importance of both the regulatory and reimbursement processes to patient access to cancer therapies, where can patients and industry focus their attention? There are many areas of mutual interest, both in terms of the regulatory process and in terms of coverage and payment for cancer therapies. Conference discussion underscores the importance of both areas in ensuring optimal patient access to new (and existing) cancer therapies, since problems in either area can prevent important services from reaching patients.

In the remainder of this section we outline areas of mutual interest identified during the conference discussion.

B. Mutual Priorities

While the link between the regulatory process and patient access seems very apparent to both industry and patient advocacy groups, the connection between reimbursement and patient access is often less direct, but can be of equal importance. Coverage, coding and reimbursement are critical to the diffusion of new oncology products where patient access problems exist. Many of the reimbursement issues discussed at the conference are joint priorities between oncology groups and industry – especially those issues where government or commercial coverage or payment systems may be slowing the diffusion of new technology to cancer patients.

Some of the issues on which advocacy groups may work jointly with industry include the following:

- **Medicare coverage of oral cancer drugs.** As new and innovative oral cancer treatments enter the market, it is increasingly important to expand Medicare coverage to include all oral anti-cancer drugs. Currently, Medicare only covers oral anti-emetics and oral chemotherapies with intravenous equivalents. This expansion would be a first step towards Medicare coverage of outpatient prescription drugs, and would ensure broader patient access for breakthrough products. Industry would also benefit from having coverage for these products for the Medicare population.

- **Ensuring consistent and fair Medicare payments across site of service.** Currently, payment levels for certain cancer therapies can vary widely among sites of service. For example, the vast majority of cancer drugs are reimbursed at much higher levels in the physician office setting than in the hospital outpatient setting. Due to Medicare underpayments for chemotherapy and other cancer drugs in settings such as the hospital outpatient setting, providers have an incentive to drive these services to the physician office. From a patient perspective, providers should choose to deliver services in the site that is most accessible and beneficial for the patient, and this decision should not be made based on payment amounts. Similarly, industry would like their products to be used in the site of service that is most convenient for the patient. Both patients and industry suffer when certain sites of service are under-paid.
- **Limiting application of authorities / creating new authorities to reduce payments under Medicare.** In recent years, Medicare has expanded or created authorities to reduce payments for drugs, including cancer drugs. Among these mechanisms are least costly alternative, inherent reasonableness, and functional equivalence. By creating and applying a wide variety of new/expanded mechanisms to reduce payments for certain drugs, the Medicare program appears to be creating financial disincentives for providers to use them. Because these authorities are not used consistently or predictably, patients, providers, and industry have limited ability to predict which products will be subject to these reductions. Therefore, financial incentives for prescribing certain products are not consistent (or even linked) to clinical benefits for the patient, and the uncertainty about application of these mechanisms can stifle industry innovation, possibly limiting the number of cancer drugs to reach the market.
- **Limiting Medicare coverage for FDA-approved indications of oncology products.** A recent Medicare trend is of concern to both patients and industry. Medicare has recently internally generated national coverage reviews of two oncology products, Zevalin and oxaliplatin, for their FDA-approved indications. In the past, Medicare generally covered oncology drugs for their FDA-approved indications, without a formal review process. Any coverage limitations related to an off-label indication for which there was limited published evidence of efficacy. This is a dangerous precedent for both patients and industry, because it could limit Medicare cancer patient access to products even after they have obtained FDA approval. No decisions have yet been published, but this is clearly an area that merits attention and monitoring by both patient groups and industry.

Although there are clear areas of overlap between industry and advocacy group interests, there are also some areas where patient and industry group interests diverge, and where priorities differ. For example, views often differ on controlling drug prices. Patient advocacy groups generally focus on the direct impact of prices on patient groups and therefore want prices to be as low as possible, to ensure the widest possible access for all patients. In contrast, industry is less favorable towards price controls that limit revenue streams and could adversely impact the ability to bring new drugs to market.

Similarly, although certain issues may be important to both the advocacy community and industry, the order of priority of these issues may not be consistent. For example, while Medicare payment for drugs in the hospital outpatient setting is of critical importance to

industry, the advocacy community is more immediately concerned with Medicare coverage of oral anti-cancer drugs.

V. How Industry and Oncology Groups Can Work Together

A. Convening Relevant Industry and Patient Organizations

Patients and advocacy groups clearly share an interest in increasing access to cancer treatments. In this context, it is possible to identify mutual priorities across the reimbursement spectrum, including coverage, coding and payment:

- **Coverage:** When payers act to limit coverage to cancer treatments, patients may not have access. A current example is Medicare's national coverage review of oxaliplatin, a colorectal cancer treatment. CMS is evaluating the drug to determine whether Medicare should cover it for its FDA-approved indication.
- **Coding:** Coding is important in that it enables providers to fill out claims and get paid for services. Delays in assignment of appropriate coding can mean that providers don't get paid for a new service, deterring use. Currently, it takes more than a year to obtain appropriate coding for new chemotherapies.
- **Payment:** When providers are paid inadequately for services, they can't afford to provide them. Both patients and industry suffer when payment is inadequate to cover the cost of services. Technologies that are underpaid in the long-term may be withdrawn from the market, even if they offer clinical benefits to patients.

There are a number of existing venues for collaboration on these issues. Regardless of the venue, by focusing on issues of mutual concern, patients and advocacy groups can leverage their influence to achieve change.

B. Educating on Reimbursement Issues

Reimbursement issues are often very complex, and many patient groups have not engaged in them significantly to date. Because the impact of reimbursement issues on patient access is not always direct, education is needed to demonstrate the links between access to care and reimbursement policy. Education is further needed so that the advocacy community can better understand how to monitor reimbursement policies of both public and private payers to ensure that they do not inhibit patient access to beneficial treatments. Because many reimbursement policy decisions that have a direct impact on patient care occur at the local level, grassroots focus on these issues is essential to both identify and combat undesirable payer decisions. By empowering the grassroots advocacy community, many reimbursement issues can be avoided before they reach the national level.

In terms of providing appropriate education on reimbursement issues to the advocacy community, it is necessary to educate not only the senior staff of patient advocacy organizations, but also organization Boards and the grassroots, so that they can feel comfortable with

terminology and tactics. Maintaining an appropriate level of education and awareness will require a sustained effort over an extended period of time.

C. Presenting Data/Concepts to Payers and Regulators

When patients and industry work together, it is important to develop appropriate dissemination strategies. The type of information presented to payers, regulators, and lawmakers may differ depending on the audience as well as who is delivering the message. For example, certain types of information will have greater credibility coming from patients as opposed to industry. Likewise, certain types of product-specific data, for example clinical trial results, may be more credible coming from the industry or from another third-party source. Sensitivity towards these issues is important in presenting the most favorable message to obtain the desired results.

In addition to collaborating on priority issues, patient and industry groups should work closely together to ensure that information being presented on a particular issue is consistent and clear. This means that both groups need to ensure consistency of messages within their organizations. Often, establishing “lead” contacts on issues of mutual importance can help to ensure the consistency and accuracy of the messages delivered. Also, ongoing communication between groups ensures that each group knows who has already been contacted and the results of prior contacts. Finally, since many reimbursement and regulatory issues must be addressed over the long-term, establishing clear channels of communication ensures that messages do not become diluted over time.

D. Establishing Reasonable Expectations

Finally, reasonable expectations are essential. Reimbursement and regulatory issues are highly complex, and the stakeholders on any individual issue are likely to be numerous. Effecting change, whether through the regulatory or legislative process, is difficult and time-consuming. It is therefore necessary to establish clear priorities and realistic timelines for achieving desired changes. In addition, both advocacy and industry groups must recognize that achieving change is a long-term goal, and consistent effort over time is needed to achieve success.

As health care costs continue to rise, economic pressures on both public and private insurers will continue to increase. Clearly, fundamental health issues loom. In the future, reimbursement decisions will determine crucial factors related to patient access to cancer care, including patient cost burden / ability to pay, physician incentives to use technology, and financial return for research and development. The course is being set now for the future, in current deliberations on the shape of public programs (Medicare, Medicaid) and trends in managed care. Patients and industry must educate themselves and be engaged on these issues now, in order to ensure that both groups’ fundamental objectives in terms of access to cancer treatments are met.