

Your Best Resource in the Fight Against Cancer

On Tuesday, November 29, 2011 in Washington, DC the National Comprehensive Cancer Network® (NCCN®), an authoritative source of information to help patients and health professionals make informed decisions about cancer care, convened the *NCCN Patient Advocacy Summit: Ensuring Optimal Care for Patients* to bring together oncology patient advocacy groups, patients, provider groups, policy-makers, industry, and employers to provide education on regulatory and coverage decisions that impact how patients are treated, explore the issue of who should decide what optimal care is for patients, and to allow these groups and other relevant stakeholders to discuss strategies for meeting patients' treatment needs. The Summit featured a series of panel discussions including representation from a variety of stakeholders in attendance. The roundtables were moderated by William T. McGivney, PhD, Chief Executive Officer of NCCN.

Opening Remarks

William T. McGivney, PhD, NCCN CEO, provided opening remarks and welcomed attendees to the NCCN Patient Advocacy Summit: Ensuring Optimal Care for Patients. Dr. McGivney's comments focused on the challenge of achieving effectiveness with efficiency in health care delivery and the joint role of providers, caregivers, and patient advocacy groups in meeting the needs of patients with cancer. Dr. McGivney noted that patients and caregivers are free to choose their clinicians and their treatments to a certain extent, but when making these choices they are often asked to become epidemiologists and economists and to adjudge, not only the medical value of a treatment plan, but its economic value and consequences. Dr. McGivney stressed the importance of shared decision-making between providers and patients as increasingly complex factors impact care delivery.

Dr. McGivney also introduced the collaboration between NCCN and the National Business Group on Health, the nation's only non-profit organization devoted exclusively to representing large employers' perspective on national health policy issues, to develop tools for employers to design health insurance benefit plans for cancer care for their employees. Several recommendations based on these tools were highlighted, including limits on copays for cancer patients, especially considering the high cost many patients face with 20% coinsurance for care costing tens of thousands of dollars, the need for coverage

of evidence-based care, and the need for parity in coverage between pharmacy and medical benefits. Attendees were encouraged to look at these tools as a potential model to advance in the policy arena, with applications for both private and public health insurance, and potentially as a way to analyze existing benefits to determine how well they serve the specific needs of patients with cancer.

FDA Reform: Meeting Patients Needs

The first roundtable focused on the Food and Drug Administration (FDA) and what reforms are needed to help patients with cancer receive timely, safe, and effective care. Panelists included Scott Gottlieb, MD, American Enterprise Institute; James Pluda, MD, Merck; Sara Radcliffe, MPH, Biotechnology Industry Organization; M. Alma Rodriguez, MD, The University of Texas MD Anderson Cancer Center; Mark Rubin, MD, Florida Cancer Specialists; and Cara Tenenbaum, Ovarian Cancer National Alliance. Conversation centered on balancing uncertainty of benefit with timely access to new drugs and biologics. The panelists discussed what expertise is available to the FDA in regard to the Oncology Drug Advisory Committee, which is tasked with reviewing and evaluating data concerning the safety and effectiveness of marketed and investigational human oncology drug products, and what should constitute conflict of interest. While much uncertainty exists, the panelists agreed that every patient wants safe, effective, and affordable medications.

The panel considered reassessing what the FDA data threshold for decision making should be in regard to approving drugs for unmet medical needs, to increase patient access to safe and effective treatments. As stated by Dr. Gottlieb, “What is the threshold for decision making, how much uncertainty, at least in unmet medical needs, are you willing to tolerate to bring a drug to the market against known risks?” It was suggested that current FDA data requirements for drug approval may be more stringent than originally intended when the accelerated approval and fast track for oncology drugs were first implemented. Legislative clarification may be needed to improve the drug approval process, which could potentially be implemented during the Prescription Drug User Fee Act (PDUFA) V reauthorization currently underway.

The balance between risk and efficacy demonstrated in clinical trials and how this is interpreted by FDA was central to this discussion. Concerns were raised that the more data required of drug manufacturers to obtain FDA approval, such as overall survival data, the more expensive drugs are for patients, limiting access to care. Considerable interest was expressed in clinical trial design and the reproducibility of

studies when using small, select patient groups in trials. Promising new technologies, the use of surrogate markers, or new trial designs could help to more easily identify treatments for specific patient populations, but also may increase the cost of drugs. The panel and attendees also considered encouraging managed care organizations and other groups to work together to generate post-market surveillance data and make it available to the public in some format. Risk and efficacy data would be extremely helpful to patients looking for answers about their care.

The composition and expertise of the ODAC was discussed. The panel emphasized the need for strong patient advocacy voices to be involved in ODAC's discussions and also noted the effect of strict conflict of interest policies governing membership on the committee. There also was debate whether ODAC panels include the appropriate experts, especially when addressing rare cancers. Changes to the FDA conflict of interest policy were suggested, such as differentiating between marketing relationships and scientific relationships to allow experts with clinical trial relationships with companies to be considered for service on ODAC. It was suggested that ODAC serves to assess clinical trial design in determining whether a specific study supports the use of a specific drug, supporting non-disease experts who are experts on clinical trial design being on the committee.

The panel discussed the possibility of implementing a progressive drug approval pathway process. With a progressive drug approval process FDA could consider different types of data to reach their final decision, allowing more flexibility in trial design, while maintaining safety. Under progressive approval a study that shows more benefit than risk could be approved without a surrogate endpoint being identified. While it was noted that FDA may already have flexibility to adopt such a pathway, including this in the reauthorization of PDUFA could increase the chances of this pathway being implemented. Ultimately, increased efficiency in the FDA approval process could lead to quicker approvals and more beneficial drugs reaching patients more quickly.

Public and Private Payors: How Coverage, Reimbursement, and Copays Impact Patient Care

The second roundtable, focusing on how coverage and reimbursement impact patient care, included Joe Bailes, MD, American Society of Clinical Oncology; Al Benson, MD, Robert H. Lurie Comprehensive Cancer Center of Northwestern University; Nancy Davenport-Ennis, Patient Advocate Foundation; James Frame, MD, David Lee Cancer Center; Pam Germain, MBA, Roswell Park Cancer Institute; Len

Lichtenfeld, MD, American Cancer Society; Michelle Martin, MBA, CBS; and Sam Silver, MD, PhD, University of Michigan Comprehensive Cancer Center as panelists.

The roundtable started with discussion around what has changed in clinical practice over the years that has impacted how providers treat and interact with patients. Many panelists indicated that their centers now employ multiple people to deal with insurance issues such as billing and pre-certification and patient navigation issues. Virtually every patient must be pre-certified for therapy which can be determined in a very fast turnaround of 30 minutes, but in some cases the decision is not determined for a week. Pre-certification and authorization can be a financial drain for some practices as well as a drain on the time of physicians. Some pre-certification vendors require a discussion with a physician who is busy treating patients. Pam Germain pointed out that few insurance companies have medical directors with oncology experience yet they are questioning the expertise of practicing clinicians. The need for pre-certification can also complicate scheduling of chemotherapy infusion for patients. These changes have greatly increased expenses for most practices.

The conversation also focused on how providers and their office staff also must try to navigate the insurance situations of their patients. While a patient may present an insurance card, there can be hundreds of contracts and different pharmacy benefit managers behind that card making it difficult for providers to help their patients understand the costs of their treatment. Providers may often direct their patients to contact patient advocacy organizations to help with difficult financial situations. Nancy Davenport-Ennis explained that six years ago it took the Patient Advocate Foundation an average of 8.6 contacts to resolve a case, while in 2010 it took an average of 15.3 contacts to resolve one case. Michelle Martin also suggested that, when a patient is employed by a self-funded employer that designs their own insurance benefits, the best route of action may be to directly contact the patient's human resources manager to address coverage issues.

Along with provider's increased involvement in the financial side of treatment, patients are faced with complex clinical and financial situations. Patients with insurance are unaware of their copays and may not realize they must use specialty pharmacies for their expensive chemotherapy drugs. Patients are often unfamiliar with the pre-certification processes and may need to wait for several weeks to receive the medications that will treat their cancer. Health insurance for many patients only guarantees them "a ticket to enter the healthcare arena", but does not guarantee affordable coverage. Panelists mentioned

the multitude of restrictions patients may come across in accessing healthcare. Patients may be restricted to a certain number of visits per year, surgical interventions, and number of prescriptions filled per month. For some patients these restrictions may be the difference between life and death. The issue of cost was discussed under the guise of whether the NCCN Guidelines should include cost information. The panel agreed that without agreed upon methodology that takes into account all the components of a given regimen, including the potential for toxicity, hospitalization, and regional differences, including cost would not be beneficial to patients and providers. Panelists also agreed that we need to understand whether knowing the cost will change behavior of providers and patients in a way that lowers cost without sacrificing quality.

Dr. Lichtenfeld broached the subject of provider responsibility and ensuring providers are giving the best care possible and are cost effective in their use of resources. Providers must be willing to demonstrate that they are delivering on this promise of best care possible in a cost effective manner. Dr. Lichtenfeld pondered “where are we going to find this transparency? Where are we going to find that willingness to step forward and say, ‘I’m willing to be publicly measured. I don’t want the government doing it. I want us to do it, and I want to demonstrate that we are doing the right thing.’” By providers demonstrating their responsibility to patients, barriers may be reduced along with administrative costs.

The panelists agreed that the resource needs of patients are large and can be overwhelming for physicians, their staffs, and patient advocacy groups to meet. These stakeholders wish to ensure that patients receive the care they need in spite of their insurance coverage and its financial implications.

Clinical Decision-Making: Who Should Decide?

Al Benson, MD, Robert H. Lurie Comprehensive Cancer Center of Northwestern University; Nancy Davenport-Ennis, Patient Advocate Foundation; James Frame, MD, David Lee Cancer Center; Ray Muller, MS, RPh, Memorial Sloan-Kettering Cancer Center; and Diana Rowden, Susan G. Komen for the Cure participated in the final roundtable of the day.

The roundtable opened with discussion about how providers discuss cost with their patients. Drs. Benson and Frame both indicated that they do discuss costs with patients, but it can be a very complicated discussion. Physicians face difficulties when discussing the costs of oral drugs with their patients due to many drugs only being available through specialty pharmacies. Dr. Frame indicated that

at his practice they start first with the patient's insurance and then move onto a charity care program. They work with copay assistance programs aggressively and when all else fails, they set up a payment plan for their patients. Dr. Frame also indicated that in his practice in West Virginia they have many patients that are first seen very late in their disease trajectory because many are underinsured and are afraid of seeing physicians.

Ray Muller spoke about his experience at Memorial Sloan-Kettering Cancer Center (MSKCC) where there are 215 pharmacists and 1500 patients a day are seen in a specialized approach to pharmacy. MSKCC has three internal retail pharmacies that can adjudicate claims for the patient before the patient goes home so they can find out if the drug is covered and how much they will need to pay. In this case, pharmacists can be very proactively involved in care decisions.

Nancy Davenport-Ennis explained how her organization, the Patient Advocate Foundation (PAF), helps in the financial situations of patients. In 2010, PAF handled about 11,000 patients that were provided direct copayment assistance with a value of approximately \$29 million. Ms. Davenport-Ennis explained that many patients faced formulary restrictions, tiered copayments that may go as high as 40%, and copays for every single radiation visit. These same patients have already leveraged equities in their homes and ran up credit bills to pay for treatment. Ms. Davenport-Ennis stated there are 7 copay programs in America today and they are all working just as hard as PAF to meet the needs of patients.

Dr. Benson explained that working with Medicare patients may be easier in terms of amount of time to figure out the financial implications of treatment, but indicated they are unable to negotiate with their local Medicare Administrator Contractor (MAC). Dr. Frame brought up the concept of how the consolidation of MACs may affect patient care with medical directors being responsible for greater and greater areas and Medicare beneficiaries. Dr. Frame stressed the need for partnerships with these people and the need for uniformity across MAC jurisdictions.

The issue of patient knowledge was also discussed by the panelists. Diana Rowden commented that when she was diagnosed with cancer she walked into her oncologist's office with a binder of materials. Her doctor took this as a sign that she wished to be involved in her treatment decisions. It is now easier for patients to get treatment information because of online resources, but this comes with limitations due to the plethora of bad information online that may mislead a patient. Ms. Rowden commented that

it is important to steer patients to appropriate, credible sources of information so that patients are prepared with questions to ask their physicians. Dr. Frame explained that more people are coming in prepared and with family members to help them in the decision-making process. This preparedness is helping patients understand the terminology doctors utilize. Dr. Frame also emphasized that it is important to ask a patient what information they would like to hear from their doctor.

In terms of patient knowledge, at MSKCC they offer 700 unique patient education leaflets (usually two to four pages of targeted information) on a multitude of topics including drugs, regimens and supportive care medications. These leaflets are written at a sixth to eighth grade level to help ensure comprehension. An additional unique offering at MSKCC is that their patient education software in the pharmacy is immediately translatable into 17 different languages to help patients that speak a multitude of languages.

Dr. Benson spoke to the importance of the patient-physician relationship in making care decisions – “I think it is important for the physician to be very clear what they think the most appropriate option is because, you know, a disease I treat all the time, colon cancer for first-line treatment for metastatic disease, there are multiple options for people. And they vary; there are different toxicity profiles. But, in the end, they’re going to want to know, ‘Well, what would be your preferred option?’ And, actually, it’s still a very common question, ‘What would you do if this was your sister or your mother?’ So, I think, particularly for brand new patients, and absolutely with a new diagnosis, there has to be this establishment of trust that there can be a working relationship; so that as a healthcare provider, you’re credible; that people believe they’re getting detailed but understandable information; and then to outline why you would have a preferred choice.”

The panelists discussed how clinical decision-making can be challenged in the face of issues such as coverage and reimbursement problems, patient knowledge, trust in a physician, and other policy issues. All stakeholders will need to continue to work together to improve the clinical decision-making process so that it takes into account the clinical authority of the providers and the financial, clinical, and emotional needs of patients.