NCCN Patient Advocacy Summit: Delivering Value for Patients Across the Oncology Ecosystem

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ABSTRACT

As the oncology ecosystem shifts from service-based care to outcomes and value-based care, stakeholders cite concerns regarding the lack of patient experience data that are important to the patient community. To address the patient perspective and highlight the challenges and opportunities within policy and clinical decision-making to improve patient-centered care, NCCN hosted the NCCN Patient Advocacy Summit: Delivering Value for Patients Across the Oncology Ecosystem on December 11, 2019, in Washington, DC. The summit featured multidisciplinary panel discussions, keynote speakers, and patient advocate presentations exploring the implications for patient-centered care within a shifting health policy landscape. This article encapsulates and expounds upon the discussions and presentations from the summit.

The United States healthcare system is transitioning from free-for-service to value-based care in the pursuit of improved outcomes and reduced costs. In oncology, the growth of precision therapy, genetic testing, and treatment innovation have led to cancer care that is multispecialty, multisite, and has multiple therapeutic options. In the midst of this changing landscape, members of the patient community are voicing concerns about the lack of inclusion and patient-centricity in the cancer care delivery system and therapeutic development lifecycle. To assess the value landscape and consider emerging challenges and opportunities related to delivering value for survivors and patients with cancer, NCCN hosted the NCCN Patient Advocacy Summit: Delivering Value for Patients Across the Oncology Ecosystem. The summit featured multidisciplinary panels, keynote lectures, and presentations from patient advocacy organizations.

Untangling Perspectives: Identifying Patient-Centered Elements of Value

As private and public enterprises seek to advance value-based cancer care in oncology, members of the patient community signal deficiencies in patient-centered elements of value in therapy development and care delivery. Specifically, advocates of the patient community cite that current patient experience data require further interrogation, because many established patient-reported outcomes (PROs) and trial endpoints do not capture what is truly important to patients or lack integration in care settings.1 Although some critics note that patient data pose challenges in measurement and lack effective intervention tools for care teams to apply, advocates affirm the patient community is equipped with tools and resources to overcome barriers and advance personalized, high-value care.1 To highlight patient community perspectives related to delivering patient-centered oncology care, NCCN convened a panel of patients and patient advocates to discuss “patient-important outcomes” in oncology and existing resources to improve their uptake in cancer care.

Financial toxicity has become a top priority for patients in the development of value-based care. According to National Health Expenditure Data, healthcare costs in the United States increased to $3.6 trillion in 2018 alone, translating to $11,172 per American. Coupled with recent Federal Reserve Board data observing that less than half of Americans may be able to afford a $400 emergency, members of the patient community increasingly want healthcare that considers and addresses patient out-of-pocket cost. Members of the patient community observe that patients with cancer are at particular risk of experiencing financial toxicity due to care costs and the potential disruptive treatment to work productivity. A study published in the American Journal of Medicine in 2018 found that >40% of patients from 2000 to 2012 exhausted all assets within 2 years of their cancer diagnosis. A similar study of 253 long-term cancer survivors discovered that only 67% were employed 5 to 7 years after treatment. “Patients with cancer are almost 3 times more likely to file for bankruptcy,” stated Carla Tardif, Chief Executive Officer of Family Reach. “Families are deciding, ‘Do I put gas in my car, or do I go to the grocery store? Without the gas, I can’t get to treatment; without food, my family doesn’t eat.’”

Sexual function and family planning is another high priority for the patient community. Although some changes are occurring, notably the expansion of fertility preservation policies in state legislatures, advocates note that needs assessments for sexual health have significant room for improvement. Queries of the patient community indicate that care team conversations about incontinence and fertility preservation still lack cultural competency. “There are men presenting with ovarian and cervical cancer. There are also women presenting with prostate cancer and testicular cancer,” stated Darryl Mitteldorf, Executive Director of Malecare, while discussing underserved communities and sexual health shortfalls. “There are 150,000 trans people on Medicare right now and almost 69,000 suffer every day because they report to us that they’re not getting cancer care that addresses their personalized needs.” Advocates also note that care teams have yet to advance fertility preservation conversations that relay all the information a patient needs to consider. An intersection rarely discussed with patients by their care teams is the ability for cancer survivors to become adoptive parents if they choose not to save reproductive tissue. A 2010 analysis of adoption agencies and state regulatory antidiscrimination protections found several policy barriers impede the ability for patients with cancer and cancer survivors to adopt.

Although encouraged by the potential of “patient-important outcomes,” such as financial toxicity and psychosocial distress assessments, other stakeholders direct attention to implementation challenges in both oncology practices and evidence generation. Currently, few community and small practices have the resources to expand wraparound services to patients. According to the ASCO’s 2017 report, “The State of Cancer Care in America,” 54% of practices surveyed identified facility expenses (staffing, equipment, overhead, rent, and administrative costs) as the primary stressor on their practice. In evidence generation, some stakeholders cite concerns about the standardization of PROs and complications associated with integration into clinical workflows. Although acknowledging these challenges, patient advocates suggest resources within their own community to resolve such issues have been overlooked. Open to Options, a decision support-counseling program developed by Cancer Support Community, helps patients consider treatment priorities and develop topics to address during care visits. Malecare, a cancer organization dedicated to advocacy for gay and bisexual men's cancer survivorship, developed a mobile application called Cancergraph to track and record survivors’ symptoms and side effects; the organization is engaging providers and pharmaceutical companies to adjust data descriptors in an effort to improve the validity of the app’s PROs.

Patient Advocate Resources and Presentations

Two patient advocacy organizations offering solutions to the advancement of patient-important outcomes are LUNGevity and the Leukemia & Lymphoma Society (LLS). Focused on soliciting patient preferences and patient decision-making data, LUNGevity launched the Patient-Focused Research Center (Patient FoRCe) in 2017. The latest study produced under Patient FoRCe, Project Transform, surveyed >900 patients with lung cancer to understand the intersection between side effects and treatment preference. Preliminary data from Project Transform reveal that patients are willing to increase the severity of their short-term side effects in exchange for 6 months of additional progression-free survival (PFS). Conversely, patients are willing to sacrifice 3 months of PFS if their treatment can be administered orally rather than via intravenous injection. Although these patient insights are compelling, Andrea Ferris, President and Chief Executive Officer of LUNGevity, emphasizes that involving other stakeholders such as industry partners, regulators, and clinicians in the study framework was essential to overcoming trepidations about data validity. “It is very important to conduct these studies in Institutional Review Board-approved ways that can then be codified and published so that they can be referred to by clinicians, regulators, politicians, and others as well.”

For LSS, recognition of financial toxicity issues has led to increased policy activity around reducing patients’...
financial burden. LLS finds the insights from its patient population well suited to address this burden. In the face of unprecedented innovation, with one-third of oncology drugs approved by the FDA in the past 5 years being treatments for blood cancers, blood cancer is experiencing some of the most significant innovations and accompanying cost concerns in oncology.\textsuperscript{13} Patients with chronic lymphocytic leukemia (CLL) enrolled in Medicare currently face out-of-pocket costs as high as $29,984 within the first 48 months of diagnosis.\textsuperscript{14} Prospective studies paint a more concerning picture: forecasting the lifetime costs of CLL treatment will increase to $604,000 by 2025.\textsuperscript{15} In response, LLS released a series of policy recommendations aimed at lowering the cost of cancer care while maintaining quality. Policy recommendations within the proposal include passing drug parity legislation to ensure patients pay an equal cost-share for an oral medication as for intravenous therapy; parity legislation to ensure patients pay equal cost; and removing impediments to value-based agreements by reforming Medicaid best-price and antikickback regulations.\textsuperscript{13,16}

**Incorporating the Patient Voice Into the Evidence**

Patient advocates’ calls to incorporate patient-important outcomes into evidence development have steadily increased with the improved capture and quantification of patient experience data. Customarily focused on assessing the efficacy and tolerability of therapeutics and medical devices, the FDA is deploying several initiatives to promote patient-focused drug development (PFDD) for oncology drugs and biologics. Many of these initiatives are housed within the recently created Oncology Center of Excellence (OCE).\textsuperscript{17} Authorized in 2017, OCE centralizes efforts by the Center for Drug Evaluation and Research (CDER) and Center for Biologics Evaluation and Research (CBER) to expedite review of oncology-related products and ensure consistency in determinations.\textsuperscript{18} Dr. Paul Kluetz, Deputy Director of the OCE, provided the summit morning keynote, highlighting the initiatives housed within OCE, as well as CBER and CDER, to incorporate the patient voice into evidence development.

Amendments to the Federal Food, Drug, and Cosmetic Act in 1962 created the traditional approval framework for disease treatments. Commonly referred to as the Kefauver-Harris Amendments, these changes created a pathway necessitating substantial evidence of drug efficacy and safety for clinical approval.\textsuperscript{19} Therapeutics must prove prolongation of patient life or improvement of quality of life through clinical endpoints or established surrogates. Directly measuring overall survival, symptoms, or function, these commonly used clinical endpoints include tumor measurement, disease-free survival, objective response rate, complete response, and PFS. These clinical endpoints are the gold standard for clinical approval because of their low risk for bias or misinterpretation.\textsuperscript{16}

Nevertheless, the advent of personalized medicine have made traditional review processes unsustainable. In the therapeutic pipeline, development of targeted therapies and immunotherapies are outpacing standard review, with 40% of CDER’s investigations covering the cancer therapy space. The rapid expansion of novel therapies, resulting in more treatment options than ever before, is prompting stakeholders to request more patient experience data from investigators and pharmaceutical companies to help patients and providers distinguish between alternatives. The expansion of somatic and germline testing has inadvertently shrunk the number of potential patients for generating trial evidence.\textsuperscript{16} “With next-generation sequencing, we have many smaller diseases,” notes Dr. Kluetz, “ROS1 mutation is seen in 3% of patients with lung cancer. You have a pool of 150,000 or 200,000 patients in the United States and suddenly you are down to a few thousand. It just becomes much harder to accrue trials.”

Authorized by the enactment of the 21st Century Cures Act, OCE and the FDA have employed several regulatory policies to resolve challenges to PFDD. A useful tool in keeping pace with innovation has been the Accelerated Approval Program (AAP).\textsuperscript{20} Created in 1992 during the HIV epidemic, this expedited approval accelerates the delivery of safe, novel treatments for severe and life-threatening illnesses. Instead of traditional endpoints, which require considerable trial lengths to yield, AAP uses surrogate endpoints that are reasonably likely to predict decreased irreversible morbidity or mortality for the patient.\textsuperscript{17,21} Recognizing the benefits of placing patients in the entire development cycle, OCE has also hosted >25 disease-specific PFDD meetings to gain more insight into the patient perspective on drug development and treatment outcomes.\textsuperscript{17} The FDA is also collaborating with ASCO to review eligibility criteria for cancer trials. “Eligibility criteria are very narrow, and oftentimes they were built on old cytotoxic chemotherapy sorts of protocols,” noted Dr. Kluetz. “We really reviewed those carefully and broadened some eligibility criteria...We know that there are trial access and disparity problems with trials.”

Another strategy the FDA is using is the improved communication of PRO data received from investigators. OCE is working with stakeholders to create interpretable visualizations of clinical trial PRO data for patients and physicians.\textsuperscript{16} In an international effort, the FDA is collaborating with the Setting International Standards in Analyzing Patient-Reported Outcomes and Quality of
Life Endpoints Data (SISAQOL) Consortium to standardize PRO analysis and interpretation. The agency has also expanded the role of labels in relaying data by incorporating PROs into the label. Since 2013, with the inclusion of visual side effects on the label of crizotinib, a treatment of advanced non–small cell lung cancer, the FDA has been steadily expanding the use of PRO data on product labels.\textsuperscript{16}

Efforts to improve the capture and use of patient experience data continue to expand across CBER, CDER, and OCE. Recent requests for feedback have touched on a number of areas to address patient-centered evidence development, including decentralizing clinical trials and utilization of digital health technology for real-world data.\textsuperscript{16} In December of 2018, the agency expanded their expertise by announcing the appointment of Dr. Amy Abernethy, former Chief Medical Officer, Chief Scientific Officer, and Senior Vice President for Oncology at Flatiron Health, to Principal Deputy Commissioner of Food and Drugs. To communicate PRO information collected in trials and analyzed during approval, the OCE has also launched Project Patient Voice, a pilot website that will make patient symptomatic side effects from trials publicly available.\textsuperscript{16}

**High-Value Cancer Care: What’s in it for Patients, Clinicians, Hospitals, Industry, and Payers?**

In recent months, lawmakers have introduced a flurry of legislation and regulation on drug costs as a panacea for lowering cost of care for patients and providers. Although important, therapeutic cost is one of many sites of care where costs can be saved. A considerable threat to high-value care, that harms all stakeholders in the oncology ecosystem, is the misappropriation of resources.\textsuperscript{22} Wasteful healthcare accounts for 30% of healthcare spend per year, costing American patients, payers, and providers billions in overtreatment, undertreatment, and low-value care.\textsuperscript{23} Dr. Justin Bekelman, Director of the Penn Center for Cancer Care Innovation at the Abramson Cancer Center, delivered the 2019 Patient Advocacy Summit afternoon keynote, providing a provider perspective on the opportunities that health systems and physicians have to improve high-value, patient-centered care and what is at stake for patients, clinicians, hospitals, industry, and payers.

A challenge to reducing healthcare costs and improving patient-centricity is the growing accountability of physicians and practices to gather and report data.\textsuperscript{22} Although essential, providers note that the need to input patient-level clinical information is overwhelming practices and accounting for a significant amount of financial and human resources.\textsuperscript{24} Furthermore, pressures to capture data in the electronic health record (EHR) take providers’ attention away from patients during conversations, leaving patients hesitant to disclose adverse effects and increasing the probability of unaddressed complications and costly emergency department visits.\textsuperscript{22}

“We’ve lost the ability to listen to patients, to capture the patient voice,” stated Dr. Bekelman, “There’s a serious disconnect.”

To enhance providers’ ability to engage in shared decision-making interventions, it is essential that oncology practices make data sharing and collection frictionless by streamlining electronic data capture and providing innovative ways for patients to disclose treatment concerns. At the Abramson Cancer Center, barriers to patients’ communication of treatment experience were assuaged by reforming the patient mobile application and expanding phone capabilities.\textsuperscript{22} “When we tried to interact with patients through an app connected to our health system that required a sign in, there wasn’t much uptake,” noted Dr. Bekelman. “But when we integrated the same technology into texting, it allowed patients to interact with their care team. Nearly every patient was thrilled. It was easy.” Abramson would eventually go further by introducing an augmented chatbot to its texting technology and permitting patients to respond regarding how they were feeling or with symptoms through text message. “It is just the beginning of a solution,” acknowledged Dr. Bekelman, “but we saw dramatic changes that signal something big. Phone calls to our health system plummeted because patients started interacting through the chatbot. A care team member is always monitoring the chatbot and takes over when the chatbot is unable to manage an appropriate response.”\textsuperscript{22}

Another barrier to high-value care for patients is suboptimal care. Specifically, the overutilization, underutilization, and inappropriate use of health services. In oncology, 75% of patients with cancer are hospitalized within their first year of diagnosis, and 16% are hospitalized more than 3 times in that year. Moreover, 70% of patients on Medicare have ≥1 emergency department visit within the first 6 months of their diagnosis.\textsuperscript{22} Many of these visits are unnecessary, unplanned, and can be avoided through appropriate therapeutic intervention. According to a 2019 study, overtreatment or low-value care account for an estimated $75.7 billion to $101.2 billion in unnecessary expense alone. “Our work with one of the national payer’s shows that only 6 in 10 enrollees receive evidence-based cancer drug regimens,” noted Dr. Bekelman. “The rest are potentially receiving suboptimal care. We can do better.”\textsuperscript{22}

Reducing suboptimal care, and therefore reducing suboptimal care costs, can be achieved through adherence to nationally recognized, continuously updated, clinical guidelines. Studies have shown that adherence to guidelines such as the NCCN Clinical Practice Guidelines
in Oncology (NCCN Guidelines) not only improves patient outcomes and reduces disparities, but also reduces costs for health systems and patients.25,26 In a 2019 study of patients with metastatic breast cancer, guideline-discordant care cost patients $1,841 more in out-of-pocket costs compared with guideline-concordant treatment (median cost responsibility, $7,421 vs $5,171, respectively).27 The oncology community may also use clinical practice guidelines to resolve tertiary issues of cost, such as freeing up monies for essential research and treatment innovation.22 “By reducing suboptimal care, we can make room for the new; transformational cell and gene therapies that are coming down the pike. Reducing suboptimal care is also in the interest of industry.” In practice, public and private payers can incentivize guideline use by streamlining reimbursement and administration processes for guideline-concordant care. In the North Star State, such an agreement was created between Blue Cross Blue Shield of Minnesota and Minnesota Oncology Network. As an alternative to prior authorization, providers within Minnesota Oncology Network are given automatic approval for prescribed treatment regimens adherent with NCCN Guidelines.28

**Overcoming the Hurdles – Integrating the Patient Voice into the Care Continuum**

Incorporating the patient voice into the cancer care continuum is a priority for many key stakeholders, including advocacy groups, physicians, and payers. Through the evolution of PROs, both key challenges and best practices have emerged. One major challenge identified by stakeholders is the availability of tools for measuring and tracking shared decision-making, care planning, social determinants of health, and overall documentation of the patient experience.29 At a minimum, tools must be validated, implemented, and integrated into technology and clinical workflows.30 Optimally, these tools would also provide useful or actionable feedback to patients and their caregivers.24 Several pilot programs have demonstrated success; however, the required resources for implementation and integration cannot be ignored. To consider solutions and assess the challenges to integration, NCCN convened a panel of providers, patient advocates, hospital administrators, and research funders to discuss how PROs can be effectively integrated into care.

Stakeholders suggested that cancer programs that have successfully implemented the patient voice may serve as inspiration to practitioners with less experience. An opportunity highlighted by panelists was the team approach. They noted that resources required for success may include specialists, social workers, and/or health technology, which may be out of reach for rural and community practices where 85% of patients with cancer receive initial care.31 Value models often attribute patients to a specific physician; however, it is important that entire teams are empowered and held accountable when implementing these programs. Additionally, technology pays an important role, and should be simple to use and nimble within clinical workflows. Finally, and perhaps most importantly, patients should remain the central focus by measuring and reporting what is truly valuable to them.

Financial toxicity is a common concern among patients with cancer, and therefore represents a prime opportunity to incorporate the patient voice into the care experience.32 According to Alan Balch, Chief Executive Officer of the Patient Advocate Foundation, for the patients they serve, knowing the financial burden and providing access to financial assistance to manage it outranks all other goals of care.24 At the Cleveland Clinic, measures taken to address the financial burden of cancer for their patients include employing financial navigators and holding biweekly huddles to discuss new patients, which include representation from the revenue cycle team, clinical team, and administrators. Kim Bell, Administrator of Cancer Services at the Cleveland Clinic Taussig Cancer Institute, highlighted that the program is scalable, because it relies mostly on shifting interaction between the existing care team and can also be successful using virtual meetings.24 Programs like this can also be expanded to incorporate a high-level review of billing practices that lead to financial burden, including consequences such as pathway removal for companies with poor billing practices. Panelists stressed that financial toxicity can be addressed from many angles, and high-impact areas for smaller practices to begin addressing financial toxicity include transportation and pharmacy costs.24

At a system level, patients must be valued participants to all aspects of cancer care and delivery, including involvement in clinical trial development. Fight Colorectal Cancer (Fight CRC) tackles this need through their Research Advocate Training (RAT) program, which trains volunteer patient advocates to be comfortable participating in cancer care conversations at all levels. The Patient-Centered Outcomes Research Institute (PCORI) welcomes participation from patients, involving >600 patients in merit review of grants and funding decisions.24 This model helps to ensure true patient needs remain at the forefront of research decisions, driving change from the ground up.

Ultimately, “if you think something is important, you have to pay for it, and you have to put it into reimbursement” according to Dr. Bekelman. Commercial and government payers, including the Centers for Medicare & Medicaid Services (CMS), can be leaders in emphasizing the value of the patient voice across the
continuum of care. The Oncology Care First model proposed by CMS would require the use of electronic PROs, which panelists agree could be a great first step. However, the overall impact of this program is dependent on practice participation, and therefore Dr. Bekelman and others caution against rigid and complex technology requirements mostly unattainable for smaller practices.

According to Dr. Balch, all patients need to be heard to “increase the likelihood that you’re going to get the right care, to the right patient, at the right time.”

**Conclusions**

Innovation, in all facets of cancer care, will only become more personalized. In response, payers, providers, and pharmaceutical companies must work with the patient community to ensure that patient experience data and the need for tumor and treatment research are balanced with the concerns of patients and to provide effective intervention tools. At risk for the oncology community is not only patient experience data that can improve innovation but also insights that can improve outcomes and lower overall system costs. Patient advocates are not concerned about the patient community’s ability to improve high-value healthcare; contrarily, they note that their advocacy organizations already house data and tools that can help improve care. “We have very serious, validated tools. We know how to do this, and we know how to find out from our patients what matters to them,” concluded Elizabeth Franklin, Executive Director of the Cancer Policy Institute at the Cancer Support Community. “We use the excuse that it’s messy, but so is the life of a patient with cancer.”

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