Advancing a Comprehensive Cancer Care Agenda for Children and Their Families: Institute of Medicine Workshop Highlights and Next Steps

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Abstract: This article highlights key findings from the “Comprehensive Cancer Care for Children and Their Families” March 2015 joint workshop by the Institute of Medicine (IOM) and the American Cancer Society. This initiative convened more than 100 family members, clinician investigators, advocates, and members of the public to discuss emerging evidence and care models and to determine the next steps for optimizing quality-of-life outcomes and well-being for children and families during pediatric cancer treatment, after treatment completion, and across the life spectrum. Participants affirmed the triple aim of pediatric oncology that strives for every child with cancer to be cured; provides high-quality palliative and psychosocial supportive, restorative, and rehabilitative care to children and families throughout the illness course and survivorship; and assures receipt of high-quality end-of-life care for patients with advancing disease. Workshop outcomes emphasized the need for new pediatric cancer drug development and identified critical opportunities to prioritize palliative care and psychosocial support as an integral part of pediatric cancer research and treatment, including the necessity for adequately resourcing these supportive services to minimize suffering and distress, effectively address quality-of-life needs for children and families at all stages of illness, and mitigate the long-term health risks associated with childhood cancer and its treatment. Next steps include dismantling existing silos and enhancing collaboration between clinical investigators, disease-directed specialists, and supportive care services; expanding the use of patient-reported and parent-reported outcomes; effectively integrating palliative and psychosocial care; and clinical communication skills development. CA Cancer J Clin 2016;66:398-407. © 2016 American Cancer Society.

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Introduction

Pediatric cancer’s interdisciplinary team-based approach to care represents the underpinning of most of modern oncology and future progress.1 It is an area of oncology that has seen remarkable results, with therapeutic advances that have increased survival rates for some cancer types, but it also presents devastating challenges in which some pediatric cancers remain incurable.2,3 Even in cancers that are considered curable, many children still experience undue suffering,4,5 and treatment toxicities cause late effects and lasting harm in the majority of survivors.6,7 Cancer-directed treatment is often still separated from supportive care services in most settings despite clear and compelling evidence calling for their integration.8,9 In addition, families coping with childhood cancer often are left impoverished emotionally, physically, and financially.4,10

Many oncology clinicians have not received sufficient primary palliative care skills training in basic pain, symptom management, and person-centered, goal-directed communication11 to equip them for reliably meeting families’ extensive quality-of-life–focused needs.8,12,11 Access to specialized pediatric palliative,
psychosocial, or rehabilitation supportive care services in hospitals, clinics, and the community is inconsistent, under-resourced, poorly or not reimbursed, and sometimes unavailable.\textsuperscript{11,14,15} Supportive care access limitations are particularly concerning as the numbers of long-term childhood cancer survivors experiencing late effects and life-threatening chronic conditions in adulthood continue to rise. With 500,000 adult survivors of childhood cancer anticipated to be living in the United States by 2020,\textsuperscript{6} caring for this growing population’s quality-of-life needs will clearly become a larger part of every practitioner’s job. Equally concerning in the nearer term is emerging evidence indicating that nearly 30\% of families experience food, energy, or housing insecurity during the first 6 months of children’s cancer treatment—confounding factors that may contribute to unfavorable pediatric cancer outcomes and raise challenges that reach beyond the health care system to resolve.\textsuperscript{16}

To examine specific opportunities for optimizing care that supports survival with high quality of life across the full life spectrum against the backdrop of these challenges, the National Cancer Policy Forum of the Institute of Medicine (IOM) and the American Cancer Society hosted a joint workshop on “Comprehensive Cancer Care for Children and Their Families” in Washington, DC, on March 9 and 10, 2015. This initiative brought together more than 100 multidisciplinary experts, funders of research, pediatric cancer advocates, and members of the public that don’t often sit together in the same setting to think about shared challenges, objectives, and steps forward. Participants included clinicians and researchers in pediatric oncology, palliative care, psychosocial support, and survivorship care representing medicine, nursing, social work, child life, and other disciplines involved in the care of children with cancer, along with representatives from the US Food and Drug Administration, the National Cancer Institute, the Children’s Oncology Group (COG), pharmaceutical companies, parent and family stakeholders, and patient advocacy organizations.

Unlike IOM consensus studies that produce reports with recommendations, this IOM workshop followed its less formal stand-alone workshop process\textsuperscript{17} to develop and deliver a summary report of its proceedings. The summary synthesizes the workshop presentations, participant input, and practical guidance provided as action steps for optimizing quality-of-life outcomes and well-being for children and families during and after pediatric cancer treatment and across the life spectrum.\textsuperscript{4} To prepare for this 1.5-day workshop, an ad hoc planning committee was formed 8 months in advance. Members were selected based on their ability to contribute balanced and diverse perspectives as parents of pediatric patients (2 members); experts in pediatric oncology (3 members), adult oncology (3 members), and supportive care (3 members); and government agency experience relating to drug development and research (2 members). Guided by a statement of task developed collaboratively by the National Academies’ staff and sponsors describing the specific questions to be addressed, the planning committee organized the agenda, identified topics, and invited a total of 35 speakers and moderators to facilitate robust workshop discussion and cross-disciplinary points of view for enhancing pediatric cancer research, clinical care, and outcomes. The IOM’s “Comprehensive Cancer Care for Children and Their Families” Web site includes a downloadable report of the complete workshop proceedings and presenting speaker slides.\textsuperscript{4,18}

During the workshop, participants heard expert presentations on the evidence behind the challenges specific to pediatric cancer and discussed potential solutions for improving research and development, quality of care, and outcomes, emphasizing the particular needs of children and families that would build on recent findings described in three related IOM initiatives: Identifying and Addressing the Needs of Adolescents and Young Adults with Cancer\textsuperscript{19} and two adult-care–focused consensus reports, Delivering High-Quality Cancer Care: Charting a Course for a System in Crisis\textsuperscript{20} and Dying in America: Improving Quality and Honoring Individual Preferences Near the End of Life.\textsuperscript{21} Family perspectives embedded as part of each session provided examples of specific lived experiences.

This article, which was prepared by a multidisciplinary subset of the IOM workshop planners and participants, highlights key workshop findings featuring real-world perspectives, actionable solutions, and suggested next steps for integrating quality-of-life priorities in pediatric research, drug development, treatment, follow-up care, and data collection. Figure 1 was developed for this article to capture the essential elements for providing comprehensive, quality cancer care for children and families discussed in the workshop and to illustrate a framework for their integration across the continuum. Themes and associated action steps are summarized in Table 1, and more details that follow the flow of the workshop sessions are provided in the sections below.

**Workshop Findings Regarding the Impact of Pediatric Cancer’s Unique Features on Treatment Advances**

Childhood cancer care involves a uniquely integrated model of collaborative, multicenter, clinical and translational research that is reinforced through a highly effective national clinical trials infrastructure.\textsuperscript{2} While workshop panelists and participants agreed that the expanding focus on studies of survivorship and the informative data guiding
Current and future clinical investigations are strong markers of progress in the field, they also acknowledged that the relative paucity of new drug and biologic products to treat the various types of cancer occurring in children is multifactorial, long appreciated, and inadequately addressed. Attempts to address efforts to improve the quality of pediatric cancer care by preventing known delayed or persistent adverse effects of successful therapy mandate a focused effort on new drug discovery and development specifically for children with cancer. Historically, despite differences in the biology of cancers affecting adults and children, therapeutic product development for children has highly leveraged adult drug discovery and development. Challenges to pediatric cancer drug development include the low incidence of the cancers typically observed in children, sample size constraints for clinical trials, the impact of normal physiologic development on pharmacologic considerations, differing perspectives on benefit-risk assessment, and a prevailing industry research and development model that is not favorable to small, albeit life-threatening, disease populations.

Genetic mutations and amplifications disrupting key signaling pathways responsible for proliferation in certain cancers have emerged as validated targets for drug development, and more than 50 molecularly targeted drugs have been approved for advanced-stage adult cancers based on favorable benefit-risk considerations, although none have yet been demonstrated to be curative. To date, precision medicine and personalized therapy have largely benefited adults, although some targeted agents have demonstrated activity in some pediatric tumors, and others likely may be relevant to specific cancers in children given their molecular pathogenesis. Even in those rare instances in which the same uncommon cancers, such as chronic myelogenous leukemia, Hodgkin lymphoma, and anaplastic large cell lymphoma, occur in both children and adults, regulatory exemption from required pediatric investigations and subsequent development for pediatric use of agents like imatinib, disatinib, nilotinib, crizotinib, and brentuximab vedotin results from orphan designation, which the IOM workshop’s industry expert panel agreed unnecessarily delays approval of and access to highly effective drugs to children for years.

Studies in childhood cancer genomics have demonstrated that childhood cancers have fewer gene mutations than adult cancers and that many childhood cancers are driven by mutations that are very rare in adult tumors. Currently, most childhood cancers at diagnosis lack the mutations in

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**FIGURE 1.** Comprehensive Quality Cancer Care Trajectory for Children and Families. The trajectory effectively integrates palliative, psychosocial, and other restorative and rehabilitative supportive care as part of disease-directed treatment and follow-up across the full continuum to reliably address cure, comfort, and quality-of-life (QOL) objectives.
| OPPORTUNITIES TO IMPROVE CARE QUALITY AND OUTCOMES | ACTIONABLE NEXT STEPS TO EDUCATE, ENGAGE, AND ENERGIZE STAKEHOLDERS |
|--------------------------------------------------|---------------------------------------------------------------|
| Strive for survival and well-being simultaneously for seriously ill children and their families | Unite professional forces across disciplines and specialties to advance cure and QOL priorities concurrently and minimize suffering so all children with cancer have opportunity to both survive and thrive |
| Enhance and accelerate pediatric cancer drug development | • Maximize use of Pediatric Research Equity Act regulatory authority by requiring studies of adult drugs in pediatric populations earlier in the drug life cycle to help correct indication-based waiver challenges |
| Increase access to early pediatric palliative and psychosocial care services in all care settings | • Create pediatric drug-development incentives that reduce industry risk earlier in the life cycle |
| Prioritize person-centered and goal-directed clinical communication | • Expand public-private and foundation partnerships as well as global research collaborations to support new pediatric drug development |
| Improve and expand the use of pediatric PROs | • Prioritize patient-centered drug development by developing instruments to accurately measure symptom reduction and enhanced function as treatment benefits, expanding focus beyond increased survival alone |
| Improve care transitions, long-term follow-up care, and survivorship outcomes | • Talk about palliative care and psychosocial support as “essential elements of optimal cancer care and quality of life” for every audience |
| | • Provide pediatric palliative and psychosocial care during and after treatment for children and their families to manage pain, distress, and symptoms |
| | • Make early psychosocial and palliative care needs screening universal in pediatric oncology |
| | • Avoid conflating palliative care with end-of-life or hospice care |
| | • Increase clinical research support for pediatric palliative care and psychosocial care studies and embed these services as part of clinical trials |
| | • Consider unique restorative experiences and supportive care opportunities available in the community for children and families through children’s oncology care camps |
| | • Expand access to specialized pediatric hospice care in all community settings and support bereaved family members |
| | • Help disseminate and implement the pediatric psychosocial and palliative care standards in clinical practice |
| | • Prioritize core professional education and training that builds generalist and specialist palliative care skills for all pediatric physicians, nurses, social workers, child life specialists, and others |
| | • Understand that honest and empathic communication is a priority in every clinician’s job and for the entire care team |
| | • Attain skills training to enhance person-centered and goal-directed clinical communication and promote available resources and trainings among colleagues |
| | • Convey hope through honest communication with families about treatment choices, prognosis, and support for their QOL priorities |
| | • Ask patients and families about what is important to them, what they are hoping for, what are their worries, and their sources of strength or comfort to help them maximize QOL |
| | • Provide a means for the child and family to report the impact of symptoms and toxicities, including psychosocial distress, and provide supportive care services addressing those symptoms |
| | • Pull stakeholders together to define and validate a core group of pediatric, adolescent, and young adult PROs that should be routinely measured |
| | • Identify opportunities to combine PROs with proxy reports and observable outcomes |
| | • Educate clinics, administrators, children, and parents on the value and effective use of PROs to inform clinical research and improve clinical care |
| | • Establish a dynamic, integrated electronic system to routinely screen children for PROs and provide real-time feedback to clinicians |
| | • Incorporate PROs and neurocognitive assessments for children in all trials, including phase 1 |
| | • Prioritize data collection, starting with core symptoms common to all cancers, and collaborate to standardize databases across different health systems |
| | • Monitor survivors early and continuously for recurrence, other cancers, and late effects, following COG and other countries’ harmonized survivorship screening guidelines |
| | • Promote professional survivorship care education and delivery using COG survivor guidelines for primary care providers and pediatric oncologists |
| | • Incorporate advanced practice nurses and other disciplines in the care of long-term survivors across disciplines and among pediatric and adult care practitioners |
| | • Assess and enhance readiness for adult follow-up care over time |
| | • Educate patients and parents about risks and need for appropriate follow-up care using a tailored and developmentally sensitive approach |
| | • Provide a succinct treatment summary and survivorship care plan emphasizing key areas of focus, such as long-term complications information, to support patient and provider education and continuity of care |
| | • Refer transitioning patients to long-term survivorship follow-up programs and standardize survivorship care |
| | • Support research to predict and prevent long-term complications, including precision medicine approaches identifying genetic variants that confer the most risk for late effects |
| | • Link existing registries and develop a comprehensive, integrated national registry to assist research studying the risks and susceptibilities of complications associated with certain therapies |
genes that are most relevant to approved molecularly targeted agents or those being developed for adult cancers.\textsuperscript{27} However, genomic interrogation of biopsy material at diagnosis as well as at relapse/recurrence may yield therapeutically exploitable information by identifying specific molecularly targeted agent(s) that may have activity in a pediatric tumor.\textsuperscript{28} Several pediatric clinical investigations to pursue this line of scientific inquiry provide evidence of significant therapeutic potential.\textsuperscript{29,30} Expanded efforts to exploit the therapeutic potential of targeting documented genomic perturbations in tumors in children using approved targeted drugs and biologic products require support and attention from both public and private sectors. In addition, some pediatric tumor genomes suggest that epigenetic aberrations may be operational in etiology or progression. The IOM panels agreed that more focused evaluation of agents affecting general and specific epigenetic regulators suggests a promising area for drug discovery and development in pediatric leukemias and solid tumors. Should these novel drugs prove effective in children, workshop participants also acknowledged that long-term toxicity assessment of these molecularly targeted agents will require new approaches to survivorship follow-up, as early adult experience has predicted different toxicity profiles compared with traditional cytotoxic agents.\textsuperscript{31}

Experts participating in the workshop confirmed that early communication between investigators, patients, sponsors, regulators, and advocates will be essential for maximizing currently existing regulatory authority to facilitate and accelerate drug development for children and for rational prioritization of the many new products developed for adult cancers to be evaluated in children. They suggested that earlier pediatric assessments of potentially promising drugs could result from modification of current legislation extending the requirement for pediatric evaluation from the identical clinical indication to the relevance of the molecular target of a new drug. Participants also emphasized that addressing the challenge of new drug development for children with cancer when no adult indication exists may require legislative changes enabling new and effective incentives for industry. The continued improvement in childhood cancer cure rates and quality of life throughout the cancer experience and survivorship require an unimpeded investment in basic and translational research to bring optimal treatments to children.

### Workshop Findings to Ensure Well-Being Along the Continuum of Outcomes

#### 1. Take Steps to Reliably Integrate Pediatric Palliative Care With Disease-Directed Treatment

Even with optimal cancer-directed treatment, approximately one in five children, adolescents, and young adults with cancer die. Furthermore, the majority of patients (>80%) who survive into adulthood nevertheless will have experienced, for some period of their cancer treatment and later in life, considerable suffering, ranging from physical pain or extremely bothersome symptoms and disabling conditions\textsuperscript{6,7,32} through to psychological, social, or spiritual suffering.

To better meet the needs of all patients with cancer and their family members, workshop participants affirmed that palliative care should be readily available to alleviate pain and other symptoms and to address sources of psychosocial and spiritual suffering. The goal of palliative care is to improve the quality of life while living with serious illness; and, although palliative care offers specific benefits to patients with progressing, advanced disease or life-threatening complications, palliative care can be beneficial to a broader range of patients and families. In addition, palliative care can often be helpful when integrated earlier in the course of cancer treatment, delivered alongside disease-directed therapy\textsuperscript{8,9,33}

Experts at the workshop outlined how palliative care can be provided in three complementary ways. “Generalist” palliative care...
care can be routinely provided by oncology team members trained to deliver basic or "primary" palliative care interventions for managing pain and symptoms and for goal-directed communication.\textsuperscript{11} If these general measures are insufficient to relieve suffering and improve quality of life to an acceptable degree, then the primary oncology team can consult the specialty palliative care service. These specialty services—consisting of an interdisciplinary team of physicians, nurses, social workers, and, at times, child life and expressive therapists, psychologists, chaplains, and other allied health professionals—can deliver more complex or extensive interventions in the pursuit of comfort and quality-of-life enhancement. Workshop participants affirmed that generalist and specialist levels of palliative care should coexist, support each other, and expand pediatric palliative care delivery into all care settings, including the home.

Hospice care services are a form of specialty palliative care delivered mostly in the home, again, by an interdisciplinary team. Several parents shared distressing experiences caring for their child at the end of life, including in their home, which underscored the workshop’s emphasis on the importance of providing access to hospice expertise specifically trained to meet pediatric patient and family needs—in whatever care setting the family chooses. This is particularly important for coping with cancers that are not responsive to treatment, when disease progression is occurring and end-of-life care is required.

Across all levels of palliative care, certain core communication tasks developed through skilled training are commonly encountered: discussing difficult prognosis information, including the possibility of death; discussing long-term treatment sequelae; working through goals of care; and making advance care plans. Evidence presented in the workshop confirmed that, if done well, discussions addressing these tasks can prepare and empower patients and families.\textsuperscript{34} The skills required to conduct these discussions effectively can be taught and developed. Training tools for clinicians, such as VITALtalk,\textsuperscript{35} Center to Advance Palliative Care,\textsuperscript{36} and End-of-Life Nursing Education Consortium (ELNEC)\textsuperscript{37} curricula; the Textbook of Palliative Care Communication\textsuperscript{38}; and other resources, are available, although none have been adapted specifically to the pediatric setting. Consistent with consensus recommendations in the IOM Quality Cancer Care and Dying in America reports and newly published, evidence-based standards of care,\textsuperscript{39} the current IOM workshop findings confirmed that providing communication skills-based training to all pediatric oncology clinicians is of paramount importance to improve patient-provider connections and quality-of-life outcomes across the cancer care experience.

Although important work has already been done regarding quality standards,\textsuperscript{39,40} the field of pediatric palliative care and hospice care needs to continue to focus on the development and dissemination of these standards. In particular, the workshop panelists and participants agreed that additional standards should address important details of the delivery of care to the patient (as well as bereavement care), skills training for the pediatric clinical workforce in generalist and specialty palliative care, and program staffing. Equally if not even more important, palliative care must implement quality assurance and continuous quality improvement.

2. Take Steps to Assess and Address Psychosocial Needs of Child and Family

Throughout the workshop, participants noted the life-changing and potentially devastating impact that pediatric cancer has on both the child and the family.\textsuperscript{41} Early factors that predict how well a family will cope with their child’s cancer include the developmental level and needs of the child, previous stressful life events and coping behaviors, early responses to diagnosis and treatment, family support and resources, social isolation, and concurrent family stresses.\textsuperscript{42-48} Over time, the family’s ability to adapt to change and remain cohesive; parent-child coping strategies; and social, environmental, and cultural variables play a key role in managing the stresses associated with childhood cancer.\textsuperscript{42,43,45,49-53}

Considerable data support the finding that most children and families are ultimately able to cope with the disease, treatment, and its aftermath,\textsuperscript{47,54-57} yet from one-quarter to one-third of children and families experience significant distress, posttraumatic stress, or other problem at some time during and after treatment, indicating a need for increased care or intensive interventions.\textsuperscript{49,54,55,58} Identifying children and families in distress is an important first step in targeting psychosocial interventions to address their unique needs. Several validated tools and trainings were featured at the workshop. The Pediatric Psychosocial Preventive Health Model identifies families at risk so that more intensive treatments are reserved for those that need it the most.\textsuperscript{59} Two screening methods, the Psychosocial Assessment Tool parent report and the Distress Thermometer (child and parent report), currently have reliability and validity data available.\textsuperscript{60,61} In addition to needed emotional support for the child and the child’s siblings, several evidence-based interventions have now emerged to reduce family distress, including Bright IDEAS, a training intervention that teaches parents problem-solving skills.\textsuperscript{62}

To fully incorporate psychosocial care as part of comprehensive care, the workshop identified several challenges that must be overcome. These include psychosocial care that is consistently available, offered, and financially supported by credentialed professionals. In addition, data are
needed that 1) identify optimal methods and timing for psychosocial screening and assessment, 2) determine whether screening leads to more effective treatments, and 3) evaluate targeted interventions based on an individual’s experience of suffering with the underlying illness along with the physical, emotional, social, and spiritual needs of the child and family. Workshop panelists emphasized that funding is critical for longitudinal studies that can inform how psychosocial needs change or stabilize during and after treatment.

Participants prioritized as an essential next step the dissemination and uptake in clinical practice of recently developed, evidence-based pediatric psychosocial standards of care to promote universal access to psychosocial support and intervention for every child and family needing these services. Moreover, participants agreed that tools like the community cancer center self-assessment matrix should be developed to support the implementation of pediatric cancer program standards and to capture data about the specific pediatric psychosocial services provided and outcomes of the care delivered.

3. Take Steps to Support Quality of Life Throughout Care Transitions and Survivorship While Also Monitoring and Mitigating the Impact of Late Effects

Expert presentations describing the latest survivorship research confirmed that the implications of cure are not trivial. Workshop presentations and discussions considered the series of evidentiary findings from the Childhood Cancer Survivor Study, which has followed 24,000 survivors since 1994 and has revealed multiple associations between childhood cancer treatments and late effects/health complications among survivors. Three-quarters of survivors experience at least one chronic condition 30 years after diagnosis, 40% of which are life-threatening or fatal. The risk for late effects increases the further survivors are from the end of treatment.

Panelists identified known risk factors for late effects (eg, treatment, dosage, age of exposure), noting that individual differences remain regarding who will experience a late effect, even with the same dosages of chemotherapy and radiotherapy. Research using a molecular epidemiology approach is helping increase the precision of late-effect risk prediction in patients. For example, comparisons of RNA and DNA specimens from survivors with and without cardiac late effects reveal genetic variants that confer risk or protection for developing heart failure from anthracyclines. Furthermore, novel interventions are underway aimed at preventing long-term complications in pediatric patients at high risk given their treatment profile. Examples discussed in the workshop included the use of β-blockers to reduce anthracycline-related heart failure and the use of low-dose tamoxifen to reduce the risk of breast cancer developing in women who received radiation therapy to the chest as young girls. Expert panelists cautioned that the science is a moving target, however, new cancer treatments bring unknown late effects, and the latency period for them to emerge can be decades. Participants agreed that developing a national registry of shared data on late effects and related factors can help advance science and the precision of identifying those most at risk.

Panelists highlighted the research demonstrating that psychosocial challenges (such as anxiety and posttraumatic stress, eating difficulties, neurocognitive late effects) and somatic symptoms (fatigue, pain) also remain or emerge as problems after treatment and can persist to long-term survival. The period immediately off treatment is an especially vulnerable time when patients and families have difficulty adjusting back to “normal” life, understanding how to manage normal noncancer-related symptoms, and living with the fear of cancer recurrence. Participants agreed that more rigorous research is needed to understand how patients and families adapt to the time off treatment and to identify those at risk for adjustment difficulties lasting to long-term survivorship.

Because of the long-term sequelae, multiple reports from the IOM and the COG recommend early monitoring for recurrence and continuous monitoring for later cancers and late effects. Despite guidelines, engagement in long-term follow-up care is a challenge, even after formal transfer to adult-based providers. Less than 20% receive risk-based, cancer-focused follow-up care, and few understand their risk for later health problems. The workshop considered a range of models of survivorship care that have been described and tested. For example, a risk-stratification approach prioritizes those who need specialized care versus those with a low risk for late effects who can be seen in the community. A shared care approach blends care between a cancer center and primary care providers. Participants agreed that enhancing transition readiness—indicators that the patient is ready and able to move on to adult-oriented care—is also critical to sustain engagement.

Overall, action steps that were identified in the workshop for optimizing survivor care and engagement will require the following: 1) educating patients and parents about risks and need for appropriate follow-up care using a tailored and developmentally sensitive approach that includes providing a treatment summary and survivorship care plan, 2) educating primary care providers and pediatric oncologists about COG survivor guidelines, 3) incorporating advanced practice nurses and other disciplines in the care of long-term survivors, 4) understanding barriers to engagement in care, and 5) assessing and enhancing transition readiness over time.
4. Take Steps to Prioritize Quality-of-Life Data Collection and Use of Patient-Reported Outcomes in Pediatric Cancer Research and Care

Acknowledging the intensity of pediatric cancer treatment and its impact on quality of life, participants agreed that integrating patient-reported outcome (PRO) measures into research and care is essential to effectively capture meaningful data about the patient experience. Several important considerations for PRO data collection were discussed.

First, participants agreed that the field should prioritize identifying when PROs should be incorporated into therapeutic clinical trials and specify a core set of PROs for use in these trials to allow comparison between studies and populations and to evaluate trends over time, as has been accomplished in adult cooperative group trials. For example, trials of de-escalation of therapy and early phase clinical trials may be appropriate settings for PRO inclusion. Participants concluded that providing a systematic and rational mechanism to include PROs in trials could also reduce the process burden, which is considerable in the cooperative group structure.

Second, the participants urged that the field should clarify the role of proxy versus self-report PROs in pediatric oncology. This clarification is particularly important for the youngest patients, because there is no valid approach to obtaining self-reports in very young or critically ill children. Differences between proxy and self-report outcomes in pediatric oncology are frequently observed. The magnitude of the discrepancy can be variable, however, and conflicting data exist regarding whether parents overestimate or underestimate their child’s health. Participants recommended that the field should prioritize bringing experts together across disciplines to identify the role for parent/guardian proxy-response in pediatric oncology and use this approach consistently in the defined setting. In addition, they affirmed that it is important to identify feasible mechanisms for child self-report to supplement parent proxy-report for very young or sick children.

Appropriate PROs must also be identified and developed for use across the adolescent and young adult (AYA) population. Evidence indicates that AYA oncology patients have not experienced the same improvements in survival compared with younger and older cancer patients, but it is not clear whether they also suffer disproportionately. One of the main challenges with understanding PROs in AYA patients is the lack of instrumentation that spans the entire AYA age range. More commonly, instruments are validated in pediatrics and adults separately. To address this gap, participants agreed that the field will need to either identify appropriate instruments for use across the relevant age spectrum or modify or create instruments that address important constructs and evaluate the psychometric properties of the modified instruments in AYA patients.

Finally, participants agreed on the necessity for implementing electronic data collection of PROs to reduce administrative burden, improve patient acceptability, avoid secondary data entry errors, enable electronic scoring, and promote better quality of data. Electronic PROs also facilitate bringing validated PROs to the patient bedside, and this PRO integration with clinical care improves patient-provider communication, person-centeredness of care, and may also improve symptom control and supportive care measures.

Conclusion

Although increasing numbers of children are living for many decades after diagnosis, the burden of morbidity, reduced quality of life, and premature mortality is substantial, increases in severity over time, and remains a persistent problem. Concerted efforts are essential to strengthen our health system and services in the community so they continuously support the needs of these children and adult survivors across their lifespan, as well as their families. As the specific stakeholder action steps identified in Table 1 reveal, this requires focused efforts on the connection between what is done in research, how that translates into clinical care of patients and support for their families, and how the field continues to engage parents and families as voices to help ensure optimal quality of life and care delivery. It also requires reframing the front-line clinical culture to reliably pair disease-directed treatment with supportive care as a conjoined “quality-of-life workforce.” Children’s oncology camps in communities across the United States routinely apply an integrated approach that seamlessly blends medical and supportive services to deliver restorative experiences valued by children and families at any disease stage.

Workshop participants emphasized that this kind of interdisciplinary integration is essential to improve survival and minimize suffering. At the same time, the field must take action to prepare the workforce in caring for adult survivors of childhood cancers who will be an increasingly large part of every practitioner’s patient portfolio.

To help advance this quality-of-life agenda through public policy, national advocacy efforts featuring federal legislation—the Childhood Cancer STAR (Survivorship, Treatment, Access, Research) Act (S1883/HR3381)—are gaining bipartisan traction on Capitol Hill with the support of an organized alliance. Through this vehicle, practitioners and parents have the opportunity to take national legislative action. We know our job is not done until every child and family receives optimal care that supports cure, comfort, AND quality of life. The children and families we serve work too hard every single day to be left with a health system delivering anything less than that.

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