ACCSCIENCE PUBLISHING

Tumor Discovery

EDITORIAL

Recent advances in pediatric cancer research: An overview

Nan Hu[1,2]*

[1]Department of Biostatistics, Florida International University Stempel College of Public Health and Social Work, Miami, United States of America

[2]Department of Family and Preventive Medicine, University of Utah School of Medicine, Salt Lake City, Utah, United States of America

(This article belongs to the *Special Issue: Current Advances in Pediatric Cancer Diagnoses and Treatments*)

Pediatric cancer is the leading cause of disease-related mortality among children, with approximately 400,000 new cases diagnosed globally each year in individuals under 20 years of age. Despite advances in therapy, survival outcomes for rare childhood cancers remain disproportionately poor, as reported by the US National Cancer Institute. This disparity highlights a critical need for continued research. Meanwhile, pediatric oncology is entering a transformative era, driven by novel diagnostic and prognostic tools, emerging therapies, and innovative approaches to prevention and intervention. Harnessing these advances provides an unprecedented opportunity to improve outcomes and reduce disparities in pediatric cancer care.

Clinical trials remain the cornerstone for advancing effective treatments, interventions, and preventive strategies in pediatric cancers. As of 2022, a total of 2,159 pediatric cancer clinical trials had been registered in the International Clinical Trials Registry Platform database.[1] Of these, 47% (1,006 trials) were conducted in high-income countries. For instance, the Children's Oncology Group, a major entity in pediatric cancer research, conducts numerous clinical trials aimed at improving treatments and outcomes for children with cancer.[2] Randomized clinical trials (RCTs) are critical in pediatric cancer treatment and prevention, especially in resource-limited settings and developing countries. A recent review paper published in *JCO Global Oncology*[3] highlights the need to expand clinical research in Africa, despite ongoing regional instability and lack of resources. The authors concluded that, while a low number of pediatric clinical treatment trials are open to African children and adolescents, clinical research of high quality is being done in Africa. Fortunately, several initiatives are fostering the development of research capacity across the continent, which is expected to enhance publication output.[3]

In contemporary pediatric cancer research, genomics and genetics studies have demonstrated significant potential to advance screening, diagnosis, prognosis, and the development of targeted therapies. For instance, multi-omics analysis shows great promise in identifying risk factors, classifying disease subtypes, and revealing targetable alterations for immunotherapy in pediatric cancers. A recent pediatric cancer study published in *Nature Communications* found that the proteome and *N*-glycans hold the potential to discover clinically relevant phenotypes and targetable pathways.[4] Multi-omics analyses have been applied to the study of Wilms tumor (WT), the most common pediatric renal malignancy. Another recent study published in *Nature Communications*[5] reported that pediatric WT harbors a relatively low mutational burden compared to

***Corresponding author:**
Nan Hu
(nhu@fiu.edu)

Citation: Hu N. Recent advances in pediatric cancer research: An overview. *Tumor Discov*. 2025;4(4):1-3.
doi: 10.36922/TD025410108

Received: October 9, 2025

Accepted: October 15, 2025

Published online: October 30, 2025

adult cancers, posing challenges for the development of targeted therapies. Nevertheless, multi-omics analysis revealed prognostic genetic alterations, distinct molecular subgroups, features of the immune microenvironment, and potential biomarkers and therapeutic targets. Multi-omics datasets from initiatives like the Children's Brain Tumor Network provide valuable insights that integrate with these models to accelerate discovery.[6] While traditional approaches—such as genetically engineered mouse models, patient-derived xenografts, and cell lines—remain essential in pediatric cancer research, innovative technologies have revolutionized this field. Genome engineering tools like CRISPR/Cas9 and organoid models now enable the study of specific genetic alterations, microenvironment interactions, and developmental pathways underlying tumorigenesis.[6] Looking ahead, the development of next-generation genomics models, such as humanized mouse systems, will be essential to accelerate functional research and translate discoveries into targeted therapeutics that aim to improve survival and quality of life for children with cancer.

Recent research has demonstrated that precision medicine can uncover novel treatment strategies for pediatric cancer. A study published in *Nature Medicine*[7] reported that precision-guided treatment (PGT), particularly when targeting gene fusions or initiated before disease progression, provided the greatest clinical benefit. The findings further showed that PGT informed by comprehensive molecular profiling can significantly improve outcomes for children with high-risk cancers. In another study published in *Nature Medicine*,[8] in which I was a co-investigator, we demonstrated that implementing functional precision medicine (FPM), which integrates genomic profiling with drug sensitivity testing of patient-derived tumor cells, can identify treatment options when standard therapies are exhausted. The study also demonstrated the feasibility of delivering FPM-based treatment recommendations to the tumor board in real-time, within a clinically actionable timeline. The implementation of precision medicine strategies is expected to become increasingly prevalent in basic and clinical pediatric cancer research, given its substantial potential to provide timely, targeted treatment regimens and personalized care to patients.

Behavioral interventions are effective in supporting contemporary pediatric cancer prevention. For example, recent research published in *JAMA*[9] reported that behavioral interventions can increase sun protection behavior among children. Although there is no consistent evidence that interventions are associated with a reduction in the frequency of sunburn in children and minimal evidence on skin cancer outcomes, interventions can increase skin self-examination. A non-RCT published in *Supportive Care in Cancer*[10] demonstrated that oral health education for children effectively reduced the incidence of oral mucositis in pediatric cancer patients. Further behavioral intervention studies are warranted to reduce the incidence of pediatric cancer, improve survival outcomes, and strengthen global cancer control efforts.

Finally, studies of disparities in pediatric cancer, including its treatment and interventions, are critical for identifying and addressing inequities in incidence, survival, access to care, and other health outcomes. For example, a study published in *Cancer*[11] found that most parents, regardless of racial or ethnic background, desire detailed prognostic information about their child's cancer. However, physicians often underestimate the information needs of Black and Hispanic parents. The study highlighted the importance of identifying parents' information preferences before prognosis discussions to better address their informational needs. By uncovering differences related to race, ethnicity, socioeconomic status, geography, and other social determinants, disparity studies can inform targeted interventions, optimized resource allocation, and policies that promote health equity.

In light of these recent advances in pediatric cancer research, we recognize the importance of developing this special issue, entitled *Current Advances in Pediatric Cancer Diagnoses and Treatments*, to showcase recent developments in pediatric oncology, including innovative treatment, diagnosis, prognosis, intervention, and prevention. Its scope encompasses a broad spectrum of studies, including clinical, translational, epidemiological, behavioral research, basic science, and genomics, highlighting the multidisciplinary nature of pediatric cancer research.

Conflict of interest

Nan Hu is an Editorial Board Member of this journal and the Guest Editor of this special issue, and declared that he has no known competing financial interests or personal relationships that could have influenced the work reported in this paper.

References

1. World Health Organization (WHO). *Childhood Cancer Drug Current Landscape and Pipeline Characteristics*. Available from: https://www.who.int/observatories/global-observatory-on-health-research-and-development/monitoring/pediatric-cancer-drug-pipeline-characteristics?utm_source=chatgpt.com [Last accessed on 2025 Sep 30].

2. Nass SJ, Balogh E, Mendelsohn J. A national cancer clinical trials network: Recommendations from the institute of medicine. *Am J Ther*. 2011;18(5):382-391.

doi: 10.1097/MJT.0b013e3181ff7e23

3. Van Heerden J, Zaghloul M, Neven A, *et al*. Pediatric oncology clinical trials and collaborative research in Africa: Current landscape and future perspectives. *JCO Glob Oncol*. 2020;6:1264-1275.

doi: 10.1200/GO.20.00159

4. Godbole S, Voß H, Gocke A, *et al*. Multiomic profiling of medulloblastoma reveals subtype-specific targetable alterations at the proteome and N-glycan level. *Nat Commun*. 2024;15(1):6237.

doi: 10.1038/s41467-024-50554-z

5. Cheng C, Zhang L, Chang X, *et al*. Integrative proteogenomic characterization of Wilms tumor. *Nat Commun*. 2025;16(1):7715.

doi: 10.1038/s41467-025-62234-7

6. Grigore FN, Yang SJ, Chen CC, Koga T. Pioneering models of pediatric brain tumors. *Neoplasia*. 2023;36:100859.

doi: 10.1016/j.neo.2022.100859

7. Lau LMS, Khuong-Quang DA, Mayoh C, *et al*. Precision-guided treatment in high-risk pediatric cancers. *Nat Med*. 2024;30(7):1913-1922.

doi: 10.1038/s41591-024-03044-0

8. Acanda De La Rocha AM, Berlow NE, *et al*. Feasibility of functional precision medicine for guiding treatment of relapsed or refractory pediatric cancers. *Nat Med*. 2024;30(4):990-1000.

doi: 10.1038/s41591-024-02848-4

9. Henrikson NB, Morrison CC, Blasi PR, Nguyen M, Shibuya KC, Patnode CD. Behavioral counseling for skin cancer prevention: Evidence report and systematic review for the US preventive services task force. *JAMA*. 2018;319(11):1143-1157.

doi: 10.1001/jama.2017.21630

10. Bezerra PMM, Sampaio MEA, Dos Santos FG, Ribeiro ILA, Santiago BM, de Sousa SA, Valença AMG. The effectiveness of an oral health education and prevention program on the incidence and severity of oral mucositis in pediatric cancer patients: A non-randomized controlled study. *Support Care Cancer*. 2021;29(12):7877-7885.

doi: 10.1007/s00520-021-06387-3

11. Ilowite MF, Cronin AM, Kang TI, Mack JW. Disparities in prognosis communication among parents of children with cancer: The impact of race and ethnicity. *Cancer*. 2017;123(20):3995-4003.

doi: 10.1002/cncr.30960