American Society of Clinical Oncology Position Statement:

Removing Barriers to Participation in Clinical Trials for Pediatric, Adolescent and Young Adult Cancer

Introduction

Childhood cancer remains a significant public health concern in the United States, despite substantial progress in its management over the last five decades.[1] The National Institutes of Health (NIH) and CureSearch report that although cancer death rates declined by 70% between 1970 and 2020, cancer remains the leading cause of death from disease for children ages 0-19.[2] The improvement of childhood cancer outcomes relies on oncology clinical trials which have contributed substantially to survival gains achieved over the past five decades.[3] Five-year survival for many pediatric cancers now exceeds 85%, reflecting the central role of clinical trials in advancing therapy and improving outcomes.[4]

Since the national clinical trials registry (clinicaltrials.gov) launched in February 2000, there have been approximately 165,000 studies registered in the United States across all diseases.[5] Of those, roughly 2,900 are registered as pediatric oncology trials.[6] Approximately 50-60% of children with cancer in the United States participate in clinical trials, far exceeding participation rates among adults with cancer. However, access to pediatric clinical trials remains impeded by structural barriers which can restrict access to innovative clinical research.[7] Additional documented barriers to pediatric participation include eligibility restrictions, and inequities tied to socioeconomic status, rurality, and other social determinants.[8] Unlike adult oncology research, which is heavily industry-funded, pediatric oncology trials in the U.S. are predominantly funded by academic institutions,

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[1] Sultan I, Alfaar AS, Sultan Y, Salman Z, Qaddoumi I. Trends in childhood cancer: Incidence and survival analysis over 45 years of SEER data. PLoS One. 2025 Jan 3;20(1):e0314592. doi: 10.1371/journal.pone.0314592. PMID: 39752445; PMCID: PMC11698462.

[2] U.S. Cancer Statistics Working Group. U.S. Cancer Statistics Data Visualizations Tool. U.S. Department of Health and Human Services, Centers for Disease Control and Prevention and National Cancer Institute; www.cdc.gov/cancer/dataviz, released in June 2024.

[3] Mikkelsen MK, Pham LTD, Sotelo C, Kelley M, Edwards M, Lion RR, Ravi H, Chantada G, Lucas JT Jr, Robinson GW, Santana V, Devidas M, Rodriguez-Galindo C, Moreira DC. The Global Clinical Trial Landscape for Children and Adolescents With Cancer. JAMA Netw Open. 2026 Jan 2;9(1):e2552510. doi: 10.1001/jamanetworkopen.2025.52510. PMID: 41493780; PMCID: PMC12776201.

[4] Sultan I, Alfaar AS, Sultan Y, Salman Z, Qaddoumi I (2025) Trends in childhood cancer: Incidence and survival analysis over 45 years of SEER data. PLOS ONE 20(1): e0314592. https://doi.org/10.1371/journal.pone.0314592

[5] National Library of Medicine. (2026, March 27). Trends and charts on registered studies. ClinicalTrials.gov. https://clinicaltrials.gov/about-site/trends-charts

[6] Mikkelsen MK, Pham LTD, Sotelo C, Kelley M, Edwards M, Lion RR, Ravi H, Chantada G, Lucas JT Jr, Robinson GW, Santana V, Devidas M, Rodriguez-Galindo C, Moreira DC. The Global Clinical Trial Landscape for Children and Adolescents With Cancer. JAMA Netw Open. 2026 Jan 2;9(1):e2552510. doi: 10.1001/jamanetworkopen.2025.52510. PMID: 41493780; PMCID: PMC12776201.

[7] O'Leary M, Krailo M, Anderson JR, Reaman GH; Children's Oncology Group. Progress in childhood cancer: 50 years of research collaboration, a report from the Children's Oncology Group. Semin Oncol. 2008 Oct;35(5):484-93. doi: 10.1053/j.seminoncol.2008.07.008. PMID: 18929147; PMCID: PMC2702720.

[8] Nathe JM, Oskoui TT, Weiss EM. Parental Views of Facilitators and Barriers to Research Participation: Systematic Review. Pediatrics. 2023 Jan 1;151(1):e2022058067. doi: 10.1542/peds.2022-058067. PMID: 36477217; PMCID: PMC9808610.

collaborative groups (like the Children's Oncology Group), or the federal government.[9] [10] Only about 16% are exclusively funded by pharmaceutical companies.[11]

Historically, it was thought that adult research could not be generalized to younger populations due to the rare and unique characteristics of childhood cancers. The necessity for dedicated pediatric oncology research is rooted in the physiological distinctiveness of children and the fact that their metabolic pathways, organ function, and metabolic rates differ, making the simple extrapolation of adult findings clinically inappropriate.[12] However, a nuanced approach to eligibility should recognize that these biological differences are not static throughout childhood. Current scientific consensus demonstrates that by age 12, adolescents reach a developmental threshold where metabolic and excretory functions align closely with those of adults.[13] Specifically, research identifies age 12—which corresponds to an approximate median body weight of 40 kg—as the point where drug pharmacokinetics and safety profiles typically become comparable to adult cohorts.[14] While it is imperative that pediatric populations are included in clinical trials, integrating adolescents and young adults (AYA) into appropriately designed adult trials is also not only scientifically sound but essential for producing robust, age-appropriate clinical evidence.

This position statement outlines a continued commitment to advancing pediatric and AYA oncology research by addressing the persistent challenges that continue to hinder research.[15]

Background

The American Society of Clinical Oncology (ASCO), a national organization representing over 50,000 physicians and other health care professionals specializing in cancer treatment, diagnosis, and prevention, has publicly worked to improve access to pediatric oncology clinical trials through collaborations, formal policy statements, and published journal articles.

Most notably, ASCO embarked on a multi-year, joint project with Friends of Cancer Research (Friends) in 2016 aimed at broadening restrictive eligibility criteria for cancer clinical trials in general, and establishing consensus recommendations to improve upon common eligibility criteria, including the minimum age for enrollment and

[9] Unger, J. M., et al. (2024). "Patient Enrollment to Industry-Sponsored Versus Federally-Sponsored Cancer Clinical Trials." Journal of Clinical Oncology.
[10] National Cancer Institute (2024). "NCI-Supported Clinical Trials Groups." *Cancer.gov*.
[11] Margaret E Macy, Elizabeth Fox, Brenda J Weigel, Jennifer H Foster, Thalia Beeles, Mary Beth Sullivan, Nicole Towcimak, Lia Gore, Malcolm A Smith, Douglas S Hawkins, US Food and Drug Administration approvals for pediatric cancer indications using Children's Oncology Group–associated trials data, JNCI: Journal of the National Cancer Institute, 2026
[12] Bavdekar, Sandeep B. Pediatric clinical trials. Perspectives in Clinical Research 4(1):p 89-99, Jan–Mar 2013. | DOI: 10.4103/2229-3485.106403
[13] Gore, L., Ivy, S. P., Balis, F. M., et al. (2017). Modernizing Clinical Trial Eligibility: Recommendations of the American Society of Clinical Oncology–Friends of Cancer Research Minimum Age Working Group. Journal of Clinical Oncology, 35(33), 3781-3787. DOI: 10.1200/JCO.2017.74.4144.
[14] Chuk, M. K., Mulugeta, Y., Roth-Cline, M., et al. (2021). Recommendations for Dose Selection for Adolescent Patients in Relevant Adult Oncology Clinical Trials. Clinical Pharmacology & Therapeutics, 111(4), 956-963. DOI: 10.1002/cpt.2458.
[15] Bavdekar, Sandeep B. Pediatric clinical trials. Perspectives in Clinical Research 4(1):p 89-99, Jan–Mar 2013. | DOI: 10.4103/2229-3485.106403

the need to prioritize enrollment of those in the AYA age gap. [16] [17] [18] [19] [20] Based on literature from the National Cancer Institute (NCI) and JAMA Oncology, the AYA population is generally defined as individuals between the ages of 15 and 39 years at the time of cancer diagnosis.[21] This age period is often described as a "no man's land" between pediatric and adult oncology.

ASCO has modeled these recommendations within its own research, most significantly through its own clinical trial platform, the Targeted Agent and Profiling Utilization Registry (TAPUR) Study. The TAPUR Study pioneered the inclusion of adolescents as young as 12, utilized pragmatic eligibility criteria, and shifted the focus from tumor-site to molecular targets.[22]

The ASCO and Friends consensus recommendations[23] led to actions by the federal government, including updates to the NCI's Generic Protocol Template and the 2020 publication of four final Food and Drug Administration (FDA) Guidance for Industry documents – most significantly, the guidance on Minimum Age Considerations for Inclusion of Pediatric Patients. This led to a shift from a "default to exclude" to a "default to include" philosophy in clinical trials.[24]

Clinical trials serve as the foundational mechanism for therapeutic advancements in cancer care and the gold standard for evaluating treatment safety and efficacy. [25] One of the most compelling reasons for dedicated pediatric oncology clinical trials is the long-term burden of morbidity carried by survivors of childhood cancer. More than 80% of children with cancer who have access to contemporary therapies are expected to survive into

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[16] Kim, E. S., Bruinooge, S. S., Roberts, S., et al. Broadening Eligibility Criteria to Make Clinical Trials More Representative: American Society of Clinical Oncology and Friends of Cancer Research Joint Research Statement. Journal of Clinical Oncology 35, 3742-3747(2017). DOI:10.1200/JCO.2017.73.7916.

[17] Lin, N. U., et al. (2017). Modernizing Clinical Trial Eligibility Criteria: Recommendations of the American Society of Clinical Oncology–Friends of Cancer Research Brain Metastases Working Group.

[18] Uldrick, T. S., et al. (2017). Modernizing Clinical Trial Eligibility Criteria: Recommendations of the American Society of Clinical Oncology–Friends of Cancer Research HIV Working Group.

[19] Lichtman, S. M., et al. (2017). Modernizing Clinical Trial Eligibility Criteria: Recommendations of the American Society of Clinical Oncology–Friends of Cancer Research Organ Dysfunction and Prior Malignancies Working Group.

[20] Gore, L., et al. (2017). Modernizing Clinical Trial Eligibility Criteria: Recommendations of the American Society of Clinical Oncology–Friends of Cancer Research Minimum Age Working Group.

[21] What Should the Age Range Be for AYA Oncology? Journal of Adolescent and Young Adult Oncology. 2011;1(1):3-10. doi:10.1089/jayao.2011.1505

[22] Mangat, P. K., Halabi, S., Bruinooge, S. S., et al. (2018). Rationale and Design of the Targeted Agent and Profiling Utilization Registry (TAPUR) Study. JCO Precision Oncology, 2, 1-14. DOI: 10.1200/PO.18.00122.

[23] Kim, E. S., Uldrick, T. S., Schenkel, C., Bruinooge, S. S., Harvey, R. D., Magnuson, A., Spira, A., Wade, J. L., Stewart, M. D., Vega, D. M., Beaver, J. A., Denicoff, A. M., Ison, G., Ivy, S. P., George, S., Perez, R. P., Spears, P. A., Tap, W. D., & Schilsky, R. L. (2021). Continuing to Broaden Eligibility Criteria to Make Clinical Trials More Representative and Inclusive: ASCO–Friends of Cancer Research Joint Research Statement. Clinical Cancer Research, 27(9), 2394–2399. https://doi.org/10.1158/1078-0432.ccr-20-3852

[24] Weber JS, Levit LA, Adamson PC, Bruinooge S, Burris HA 4th, Carducci MA, Dicker AP, Gönen M, Keefe SM, Postow MA, Thompson MA, Waterhouse DM, Weiner SL, Schuchter LM. American Society of Clinical Oncology policy statement update: the critical role of phase I trials in cancer research and treatment. J Clin Oncol. 2015 Jan 20;33(3):278-84. doi: 10.1200/JCO.2014.58.2635. Epub 2014 Dec 15. Erratum in: J Clin Oncol. 2019 Feb 1;37(4):353. doi: 10.1200/JCO.18.02274. PMID: 25512456; PMCID: PMC4516884.

[25] Shusterman, S. B., et al. (2021). Barriers to Enrollment on National Cancer Institute Community Oncology Research Program Children's Oncology Group Clinical Trials at Affiliated Sites: A Survey of Principal Investigators. JCO Oncology Practice, 17(5), e620-e628.

adulthood.[26] However, the therapies responsible for this survival can also produce adverse, long-term, health-related outcomes, referred to as late effects. Late effects such as organ dysfunction, secondary carcinogenesis and growth, and development can appear from months to years after treatment.[27] Research shows that between 60% and more than 90% of childhood cancer survivors will develop one or more chronic health conditions.[28] High quality survivorship data is needed to establish the occurrence of risk profiles for late cancer treatment-related toxicity.

Barriers to Participation in Cancer Clinical Trials for Pediatric, Adolescent, and Young Adult Populations

Pediatric Cancer Funding

One of the most significant barriers to participation in pediatric oncology clinical trials is the limited funding which impacts research priorities. Pediatric oncology trials remain dependent on non-industry sponsorships, while industry-sponsored oncology trials rarely include pediatric groups.[29] According to the NIH, only 4% of all government-allocated cancer research funds go towards pediatric cancer research, despite children comprising nearly one-fifth of the U.S. population.[30] These funding restraints limit early-phase drug development pipelines and reduce opportunities to improve pediatric cancer outcomes. For survivorship-specific research, which can last 30–50 years, industry funding is virtually nonexistent, leaving a critical research gap for the estimated 500,000 childhood cancer survivors living in the U.S. today.[31] Thus, the burden of improving long-term quality of life for survivors falls entirely on the same limited pool of federal and philanthropic funding that is already stretched thin across early-phase research.

Compared to adult research, pediatric studies receive little private investment because the markets are small, and pediatric clinical research is complex. While federal initiatives such as the Childhood Cancer Data Initiative and the Childhood Cancer Survivorship, Treatment, Access, and Research Act have launched to combat this gap, current appropriations remain insufficient to support the scale of infrastructure required across more than 200 distinct pediatric cancer types. Without a protected and predictable funding stream for more effective therapies, children and adolescents will continue to have limited opportunities to access clinical trials.

Under-Enrollment of Adolescents and Young Adults

A study by University of Colorado Cancer Center researchers working at Children's Hospital Colorado reveals a group of cancer patients that has historically and continues to under-enroll in clinical trials, namely AYA patients

[26] National Cancer Institute: NCCR*Explorer: An interactive website for NCCR cancer statistics. Bethesda, MD: National Cancer Institute. Available online.
[27] Armstrong GT, Kawashima T, Leisenring W, et al.: Aging and risk of severe, disabling, life-threatening, and fatal events in the childhood cancer survivor study. J Clin Oncol 32 (12): 1218-27, 2014.
[28] Esbenshade AJ, Lu L, Friedman DL, et al.: Accumulation of Chronic Disease Among Survivors of Childhood Cancer Predicts Early Mortality. J Clin Oncol 41 (20): 3629-3641, 2023.
[29] Neel DV, Shulman DS, Ma C, Bourgeois F, DuBois SG. Sponsorship of oncology clinical trials in the United States according to age of eligibility. Cancer Med. 2020;9:4495–4500. https://doi.org/10.1002/cam4.3083.
[30] Lee, S. (2025, November 19). Lee leads bipartisan effort to properly fund pediatric cancer research. [Press release]. Office of Congresswoman Susie Lee. https://susielee.house.gov/media/press-releases/lee-leads-bipartisan-effort-properly-fund-pediatric-cancer-research
[31] "Childhood Cancer Research." American Childhood Cancer Organization, https://www.acco.org/research/. Accessed 7 Apr. 2026.

from ages 15 to 39.[32] Unlike pediatric oncology trials, AYA enrollment rates remain below 20% nationally.[33] Unfortunately, AYA cancer patients represent a population that has failed to see the same improvements in outcomes that their younger counterparts have. This persistent gap in outcomes is closely linked to structural barriers that limit AYA access to clinical trials, including age-restricted eligibility criteria, and the fragmentation between pediatric and adult oncology care systems. Addressing these barriers is essential to improving therapeutic innovation, survival gains, and widespread research participation for this population.

Access to Clinical Trials

Access to clinical trials is primarily constrained by logistical and geographic barriers, including complex trial designs and the inherent burdens of travel. Because the majority of pediatric oncology studies are concentrated at large academic cancer centers rather than community settings where many patients receive care, a patient's ability to participate is often dictated by their proximity to these specialized hubs. For families living in rural areas or with limited financial resources, travel, lodging, and missed work reduce the feasibility of participation when trials are available.[34] These geographic and financial hurdles directly impact trial enrollment and retention, often limiting a family's ability to utilize innovative therapies. Furthermore, the intensity of modern protocol requirements - characterized by frequent multi-site evaluations and rigorous visit schedules - places an immense administrative and personal strain on caregivers. National analysis supported by NCI has also identified these structural barriers, compounded by fragmented referral pathways between local providers and research centers contributing to persistent barriers.[35]

ASCO Policy Recommendations

While ASCO has been involved with several efforts aimed at advancing access to oncology clinical trials, particularly through influential collaborative efforts in this space, substantial work remains to fully dismantle additional barriers. ASCO makes the following recommendations related to removing these barriers to participation in clinical trials for pediatric and AYA cancer:

- **Funding for Pediatric and AYA Oncology Research** – Dedicated federal pediatric research funding is essential to sustain national trial networks, accelerate pediatric specific drug development, and ensure broad access to clinical studies. Additional dedicated funding specifically for the AYA population would strengthen the AYA enrollment infrastructure to expand community site activation and improve recruitment and retention of this subset of participants.
- **Minimum Age Enforcement** – Encourage inclusion of adolescents ages 12+ in adult oncology trials when biologically appropriate to include patients in the AYA population and provide more overlap with the upper range of pediatric trials. A regulatory shift in eligibility frameworks from age-based to biology-

[32] Faulk, K. E., Simpson, M., & Tarbell, N. J. (2020). Study shows fewer kids enrolling in cancer clinical trials. University of Colorado Anschutz Medical Campus. (Original research published in Journal of Clinical Oncology).
[33] Unger JM, Cook E, Bleyer A. (2019). The role of clinical trial participation in cancer research: barriers, evidence, and strategies. Journal of Clinical Oncology.
[34] Ruiz, S., Hudson, M. M., Ehrhardt, M. J., Maki, J., Ackermann, N., & Waters, E. A. (2023). Childhood cancer survivors, financial toxicity, and the need for multilevel interventions. Pediatrics, 152(1), e2022059951. https://doi.org/10.1542/peds.2022-059951
[35] National Cancer Institute. (2024). Structural barriers to pediatric oncology clinical trial enrollment: An analysis of referral fragmentation and health disparities. U.S. Department of Health and Human Services, National Institutes of Health.

based enrollment would remove arbitrary upper and lower age cut offs that delay access to novel therapies.

- **Expanded Access** – Access to pediatric clinical trials should expand beyond academic pediatric centers, especially since most AYAs receive treatment outside pediatric specialty hospitals. NCI should use dedicated federal funding mechanisms to prioritize expansion of community site trial activation, and travel and lodging assistance for families to ensure robust participation in pediatric oncology trials. Lastly, shared referral partnerships between community providers and pediatric academic centers would strengthen trial enrollment pathways.

Conclusion

Improving patient access to cancer clinical trials is a fundamental ASCO priority because it leads to progress in cancer care and improved patient outcomes. While an analysis of the pediatric clinical trial portfolio reveals that the volume of trials is robust and participation rates exceed what is seen in the adult population, barriers persist. ASCO remains steadfast in calling to address these barriers and ensuring widespread access to oncology clinical trials for all patients with pediatric cancer.

Questions? Contact policy@asco.org