

AACR Releases First Pediatric Cancer Progress Report

More than 30 new drugs have been approved for pediatric cancer in the past 10 years, but progress has not benefited all patients.

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PHILADELPHIA – Today [December 4], the American Association for Cancer Research (AACR) released the [AACR Pediatric Cancer Progress Report 2025](#). This first-of-its-kind report highlights how research is transforming outcomes for children and adolescents with cancer. It also underscores the ongoing challenges to future progress against pediatric cancers, including gaps in our knowledge of these rare diseases and persistent disparities, as well as the urgent need for increased federal and private investments, international collaborations, and innovative research approaches to address these challenges.

AACR Pediatric Cancer Progress Report 2025

“With this inaugural report, we are highlighting the tremendous progress achieved against pediatric and adolescent cancers and are discussing the remaining challenges,” said Elaine R.

Mardis, PhD, FAACR, cochair of the report's steering committee and an AACR Past President. "The report elucidates how innovative research, often funded by the National Institutes of Health (NIH), has improved outcomes for many of our youngest cancer patients."

"We also document that in several lethal cancer types, there remains a significant need for new insights, novel therapies, and international clinical trials to achieve similar progress," she continued. "One route to address this need is by emphasizing the importance of continued data and information sharing across the world and pursuing new model systems for research, given the rare nature of these diseases. As we gain more insights into pediatric and adolescent cancers through global collaboration, it is my sincere hope that future treatments will be not just curative but also less toxic, without long-term mental and physical health consequences."

Recent Advances Against Pediatric Cancers

Decades of research, collaboration, advocacy, and legislative and policy actions have transformed the outlook for cancers affecting children and adolescents.

According to the AACR Pediatric Cancer Progress Report 2025:

- In the United States, the five-year survival rate for all pediatric cancers combined has increased from 63% in the mid-1970s to 87% in 2015-2021.
- Pediatric cancer mortality in the United States declined by 57% between 1970 and 2000, and by a further 19% from 2001 to 2023, reflecting advances in risk-stratified therapy, precision medicine, and supportive care.
- Over the past few decades, research has uncovered the unique biological underpinnings of pediatric cancers, which are rare compared with adult cancers and are biologically distinct in their cellular origins, genomic drivers, tumor types, and therapeutic vulnerabilities.
- Between 2015 and 2025, the U.S. Food and Drug Administration (FDA) approved more than 20 molecularly targeted therapies and more than 10 immunotherapies for pediatric cancers.

Indeed, the proportion of drugs approved specifically for pediatric use rose from 5.9% between 2012-2016 to 13.8% between 2017-2021. While these numbers are significantly lower than what has been approved for adult cancers, the new treatments have been transformative for certain patients.

- Advances in precision medicine not only have led to new anticancer therapeutics but also have allowed clinicians to tailor treatment intensity based on the molecular profile of a child's or adolescent's cancer, reducing therapy intensity for pediatric patients with a favorable disease profile and increasing it for pediatric patients with a higher risk of recurrence.
- Technologies such as gene editing, liquid biopsy, and artificial intelligence are deepening our understanding of pediatric cancers and paving the way for additional breakthroughs.
- The second AACR Childhood Cancer Predisposition Workshop, held in 2023, updated evidence-based surveillance guidelines for certain cancer predisposition syndromes, including recommendations to begin surveillance at birth or during early childhood, with blood tests and ultrasound or magnetic resonance imaging (MRI) prioritized over computed tomography (CT) to reduce radiation exposure.
- Several innovative international programs are encouraging collaboration across the pediatric cancer research spectrum, from sharing molecular profiling data to providing treatment for children and adolescents in low-resource settings.

Ongoing Challenges in Understanding and Treating Pediatric Cancers

Despite these promising trends, pediatric cancer is a persistent public health challenge with often devastating personal and familial consequences. In 2025, it is estimated that nearly 15,000 children and adolescents will be diagnosed with cancer in the United States, and, sadly, nearly 1,700 will die from the disease.

According to the AACR Pediatric Cancer Progress Report 2025, survivors of pediatric cancer face a significantly elevated risk of long-term health issues, with an estimated 60% to more than 90% of survivors developing at least one chronic health condition in adulthood as a result of their cancer or its treatment. By age 50, pediatric cancer survivors experience nearly twice as many chronic health conditions as their peers without a history of cancer. Pediatric cancer survivors also face financial, social, and psychological challenges, including higher rates of anxiety, depression, and post-traumatic stress compared with their peers without a cancer history.

Furthermore, the remarkable progress that has been made against pediatric cancer remains uneven across cancer types and population groups. As discussed in the report:

- Although five-year survival rates in the United States exceed 90% for some cancers—such as Hodgkin lymphoma, thyroid carcinoma, and retinoblastoma—other cancers, including certain gliomas and sarcomas, are still among the deadliest, with five-year survival rates below 25%.
- Significant disparities in incidence and outcomes of pediatric cancers persist across racial, ethnic, geographic, and socioeconomic groups in the United States. For example:

- Hispanic children have the highest cancer incidence rates in the United States.
- Non-Hispanic Black children are nearly 30% more likely to die from certain pediatric cancers than non-Hispanic White children.
- Children and adolescents living in rural or economically disadvantaged areas also face higher mortality, often due to limited access to specialized cancer centers, clinical trials, and supportive services.
- On a global scale, the disparities in childhood cancer incidence and mortality are striking:
 - Approximately 400,000 children worldwide develop cancer each year.
 - Between 80% and 90% of pediatric cancer cases occur in low- and middle-income countries.
 - Due to underdiagnosis, limited resources, and subsequent delays in care, pediatric cancer survival rates in low-income countries are much lower than in high-income countries such as the United States.
 - For example, survival rates for retinoblastoma, an aggressive eye tumor, reach up to 98% in high-income countries but fall to just 57% in low-income countries.

Overall, progress against pediatric cancers has slowed in recent years compared with progress against adult cancers. Pediatric cancers are rare, which means that there are fewer patients available to participate in clinical trials and no market incentives for companies to develop new treatments. In this context, international collaborations as mentioned above and federal support for pediatric cancer research are essential to future advances for the benefit of these young patients.

The Path Forward and a Call to Action

The AACR Pediatric Cancer Progress Report 2025 ends with a strong call to action urging Congress and leaders at federal agencies to prioritize pediatric cancer research and care.

“This first AACR Pediatric Cancer Progress Report is a celebration of the incredible progress in treating children and adolescents with cancer coupled with a call to action toward a brighter tomorrow,” said Kimberly Stegmaier, MD, cochair of the report’s steering committee. “As a

pediatric oncologist and physician-scientist, I have witnessed firsthand this transformation in care for some pediatric patients with cancer but also the heartbreak when our treatments fail. Research brings a new future for these vulnerable children, adolescents, and their families. Now is the time to come together.”

Specific policy recommendations in the report include:

- Provide robust and sustained federal funding of at least \$51.303 billion for the NIH and \$7.934 billion for the National Cancer Institute in fiscal year 2026, and increase support for the federal agencies and programs that are focused on pediatric cancer research and patient care.
- Expand access to clinical trials and promising therapies for children and adolescents with cancer through regulatory reform and programs and policies that increase clinical trial participation, such as outreach efforts to rural and socially disadvantaged areas and Medicare reimbursement models that support the costs involved with clinical trial participation.
- Modernize and evaluate current pediatric cancer research programs and policies to better support the discovery and development of treatments as well as to improve patient care.
- Support efforts that leverage and harmonize all available data to aid pediatric cancer research.
- Foster global and public-private collaborations to accelerate pediatric cancer research and treatment development.
- Strengthen survivorship and long-term care for pediatric cancer survivors by ensuring comprehensive, accessible, and reimbursable follow-up care.

“We are deeply grateful to the steering committee for its extraordinary leadership in bringing this important report to fruition, as it will serve as an essential resource for those in the medical research and policy communities who are dedicated to improving pediatric cancer prevention, diagnosis, treatment, and survivorship,” said Margaret Foti, PhD, MD (hc), chief executive officer of AACR. “By drawing attention to the current promise and persistent challenges in this field, we trust that this report will catalyze increased federal and private investments in pediatric cancer research, ensuring that children and adolescents benefit from the same advances that are transforming the cancer care of adult patients.”

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