Pediatric Cancer Landscape 2026

Policy Recommendations

The following recommendations have been adopted by the Alliance for Childhood Cancer to address barriers to pediatric cancer research and drug development. They have been developed through a multi-stakeholder process and supported by research. They address the population that includes children and adolescents aged 0-19, and represent a path forward to ensure progress. While there are many other areas of urgent need with respect to pediatric cancer, such as access to care and survivorship, these recommendations are not meant to address those issues.

Guiding Principles

- **Pediatric cancer is unique** — Pediatric cancers require dedicated research, treatments, and care models that are tailored to the unique biological and developmental status of children rather than adopting adult models. When appropriate, drug development plans should include children early in the process, especially when the particular cancer indication for which a drug is developed occurs in both adult and pediatric populations.

- **Evaluate pediatric impacts of broader policy changes and programs** — Every oncology innovation, potential investment, or policy change should include an evaluation of the opportunities and issues specific to pediatric cancers.

- **Every child matters** — Every child and adolescent deserves access to high-quality clinical trials and the opportunity to participate in state-of-the-art translational research regardless of geography, race, ethnicity, or income.

- **Rarity is an opportunity** — While the rarity of pediatric cancers is often seen as a barrier to research, the pediatric cancer community is organized in such a way that it provides an opportunity to pilot innovative research and regulatory approaches to make therapeutic development more efficient and effective.

- **Healthy survivorship is a research priority** — It is not enough to cure cancer; survivors of childhood cancer have entire lifetimes in front of them and deserve to live healthy and productive lives free from lasting side effects from cancer or its treatment. Research to reduce toxicities and interventions to prevent them from occurring are moral imperatives.

- **Research funding is critical** — Continued, sustained federal investment remains the primary means of support for pediatric cancer clinical trials infrastructure and associated research, which are critical to improving outcomes.

- **Collaboration accelerates progress** — No single institution or sector can effectively study pediatric cancer alone. Decades of advances in pediatric cancer are the result of collaborations, built together by families, survivors, organizations, clinicians, researchers, government agencies, and industry, and sustained collaboration remains essential.

- **Patient and family voices are essential** — Those with lived experience should help shape research priorities, care delivery, and policy through meaningful representation in advisory and governing boards and bodies.

Policy Recommendations

Sustain Robust Federal and State Pediatric Cancer Research Investment

While federal funding serves as the foundation supporting advances in cancer care for all age groups, it—along with some state funding—plays a more significant role for pediatric cancers because of limited industry funding relative to adult cancer indications. Sustained, robust, durable, and coordinated federal and state funding is needed for discovery to drive progress in pediatric cancer treatment. This includes both general research funding as well as targeted programs such as the Childhood Cancer Survivorship, Treatment, Access, and Research (STAR) Act, the Childhood Cancer Data Initiative (CCDI), and the Gabriella Miller Kids First Act.

- Increase and sustain federal funding of pediatric cancer clinical trial networks and include a focus on improving access in community and rural settings.
- Minimize administrative burdens of site participation in federal trial networks to encourage broad participation.
- Sustain site research payments that support enrollment of pediatric patients onto federally funded trials at levels that cover actual costs.
- Coordinate between different agencies providing federal investment in pediatric research to reduce duplication and maximize the effectiveness of resources.
- Prioritize investment in research to better understand how to improve not only survival, but also long-term quality of life for survivors of pediatric cancer.
- Coordinate on pediatric cancer data collection and patient enrollment nationally and internationally to generate sufficient evidence within these rare patient populations. Collaboration and cooperation are necessary to support efficient and timely pediatric drug development.

Learn From Every Patient

While clinical trials are a key source of data to drive advances, the experience of every patient—whether in a trial or not—can provide insight. This is especially important for pediatric cancer research, given the smaller number of children and adolescents with cancer. To learn from every patient's experience, an integrated and secure data ecosystem must be optimized to preserve patient privacy while facilitating international collaboration.

- Increase and sustain federal investment in the collection and sharing of data through interoperable, standardized data infrastructure suitable for pediatric cancer populations. Examples of such investments include the Childhood Cancer Data Initiative (CCDI) and ARPA-H's Pediatric Care eXpansion (PCX) initiative.
- Ensure research data represents the full demographic spectrum of children with cancer in the U.S.
- Protect children's and families' right to control how their data are used, and guard against unauthorized disclosure or misuse.

Advance Innovative Clinical Research and Regulatory Frameworks

Research occurs within a specialized ecosystem and regulatory frameworks that ensure scientific rigor and protect research participants from unethical treatment. Studying cancer in children and adolescents involves special considerations compared to adult cancer populations. These considerations include unique ethical issues as well as challenges related to the smaller patient population. Small population sizes can mean that relatively few children are available to take part in clinical trials to study new treatments. This rarity can create competition between trials, slow drug development timelines, and make traditional clinical trial and statistical designs difficult to undertake.

- Support modernized trial design standards that include children and adolescents in adult protocols when the cancer being studied may occur in those age groups.
- Address regulatory and administrative barriers that delay activation of trials for rare pediatric cancers.
- Promote utilization by U.S. Food and Drug Administration (FDA) of the full range of innovative regulatory approaches and tools such as Bayesian statistics and real-world control arms to accelerate pediatric drug development.
- Lower barriers for patient participation in pediatric cancer clinical trials through innovative trial design.
- Ensure ongoing international collaboration on research, regulatory requirements, and marketing submissions to FDA.

Provide Market Incentives to Drive Pediatric Cancer Drug Development

While federal funding enables research focused on basic understanding of cancer, industry funding is typically responsible for translating those findings into approved therapies available to patients. Industry invests in this research in order to realize economic returns if a therapy is ultimately approved. However, the relatively smaller population of pediatric cancer patients means significantly reduced natural market incentives for industry development of therapies compared to adult cancer indications. Without corrections, this “market failure” results in few therapies developed for children and adolescents. A limited number of public policies have been created to address these shortfalls, and in pediatric cancer, government funding can also spur innovations when industry funding is unavailable. Not only do market forces create a barrier to the development of new therapies, but they also have caused shortages of older generic drugs that are frequently used to treat pediatric cancers. Public policies can help address these challenges with additional incentives and requirements.

- Sustain incentive structures that make pediatric drug development commercially viable.
- Encourage use of public-private partnerships for the derisking of pediatric cancer drug development.
- Ensure international collaboration on systems of pediatric drug development incentives and requirements.
- Address chronic pediatric drug shortages by strengthening early warning systems, addressing market failures, and providing incentives for strengthening supply chain resilience, especially related to essential generic medicines.