February 24, 2025

The Honorable John Thune
Majority Leader
United States Senate
Washington, D.C. 20510

The Honorable Chuck Schumer
Democratic Leader
United States Senate
Washington, D.C. 20510

The Honorable Mike Johnson
Speaker
U.S. House of Representatives
Washington, DC 20515

The Honorable Hakeem Jeffries
Minority Leader
U.S. House of Representatives
Washington, DC 20515

Dear Majority Leader Thune, Democratic Leader Schumer, Speaker Johnson, and Leader Jeffries:

The undersigned childhood cancer organizations are members of the Alliance for Childhood Cancer, consisting of patient advocacy groups, healthcare professionals, and scientific organizations representing Americans who care deeply about childhood cancer. We are writing to urge you to prioritize the needs of children with cancer by making key investments in research and taking action on critical legislation in the first available legislative package of the 119th Congress.

Approximately 1 in 264 children in the U.S. are diagnosed with cancer before their 20th birthday. Unfortunately, cancer remains the most common cause of death by disease among children in the United States. Unfortunately, 1 in 5 children diagnosed with cancer in the U.S. will not survive, and for the ones who do, the battle is never over. By the age of 50, more than 99% of survivors have a chronic health problem, and 96% have experienced a severe or life-threatening condition caused by the toxicity of the treatment that initially saved their life, including: brain damage, loss of hearing and sight, heart disease, secondary cancers, learning disabilities, infertility and more. By the time a child in treatment for cancer today reaches the age of 50, we want these statistics to be far less grim.

Prioritizing a 'Childhood Cancer Package'

In the 118th Congress, the House of Representatives unanimously passed five bills that would address many of the most critical needs of childhood cancer patients. This past December, each bill was included in the bipartisan negotiated health title of the end-of-year package. Unfortunately, the final version of the continuing resolution left out each of these bills. Together, this '5-bill childhood cancer package' would have constituted the most influential childhood cancer legislation since the passing of the original Childhood Cancer STAR Act.

The 5-bill childhood cancer package would address some of the most pressing needs of children and families with cancer, including extending vital research incentives, ensuring children with cancer continue to have access to the newest cures, and removing bureaucratic red tape that

impacts access to specialty care and clinical trials. Now that the 119th Congress has been seated, it is more important than ever that Congress uses the first available and appropriate legislative vehicle to move this critical childhood cancer package over the finish line. This package includes:

Creating Hope Reauthorization Act (118th Congress: H.R. 7384/S. 4583) – Initially passed in 2012 and reauthorized through 2024, this legislation expanded the FDA priority review voucher program to incentivize pharmaceutical manufacturers to invest in drugs with indications for rare pediatric diseases. Under this pediatric priority review voucher program, a qualifying rare pediatric drug approval earns a manufacturer a voucher that guarantees them access to the FDA's priority review system for any subsequent drug review. The pediatric priority review voucher program expired at the end of 2024, and Congress must expeditiously reauthorize this program before pediatric drug development is irreversibly impacted. In the 119th Congress, the Creating Hope Reauthorization Act was also included in H.R 1262, the Give Kids a Chance Act.

Innovation in Pediatric Drugs Act (118th Congress: H.R. 6664/S. 4905) – There are close to 7,000 rare diseases without appropriate treatments, and the vast majority of orphan diseases affect children. While orphan drugs once made up only a small percentage of newly approved drugs, today, most drugs approved are orphan drugs. Unfortunately, due to an exemption under current law, FDA is not allowed to require orphan drugs to be studied in children, aside from certain oncology drugs. Further, under the Pediatric Research Equity Act (PREA), drug companies are required to study adult drug indications in children when children could benefit from pediatric studies. While sponsors can request deferrals for their pediatric study commitments, FDA's existing authorities to enforce these deadlines have proven insufficient. Current law allows FDA to assess civil monetary penalties (CMPs) for late post-market study requirements for adults, but the orphan drug exemption under PREA forbids FDA from doing the same for children.

The Innovation in Pediatric Drugs Act amends the PREA to also remove the orphan drug exemption for all drugs and address the inequity in levying CMPs by giving FDA the authority to ensure post-market pediatric studies are completed on time. The bill also provides critical funding increases to study off-patent drugs that require further research in children. Together, these policies will maximize the delivery of potential therapies to the children who need them most. Provisions from the Innovation in Pediatric Drugs Act unanimously passed the House of Representatives in the 118th Congress. In the 119th Congress, provisions from the Innovation in Pediatric Drugs Act were also included in H.R 1262, the Give Kids a Chance Act.

Accelerating Kids' Access to Care Act (AKACA) (118th Congress: H.R. 4758/S. 2372) – Children with complex medical needs such as cancer, patients are routinely required to travel out of state to receive care or participate in a clinical trial. More than half of children in the United States rely on Medicaid and the Children's Health Insurance Program (CHIP) as their central sources of health care coverage, but Medicaid and CHIP coverage is limited to providers in a child's home state. If a child's medical condition requires them to obtain care from an out-of-state provider or care team, the provider must go through the screening and enrollment process in the child's home state Medicaid program, which can cause burdensome delays in providing time-sensitive care.

The Accelerating Kids' Access to Care Act would address these delays by creating an alternative opt-in pathway for providers in good standing to enroll in multiple state Medicaid programs, allowing them to provide essential, time-sensitive care to children who need it. The legislation pertains only to provider screening and enrollment and does not change the authority states have to authorize out-of-state care or negotiate payment with providers who accept such cases. AKACA would reduce delays in providing time-sensitive care to the children most in need, reduce administrative burdens and costs, and reduce the risk of care disruption and subsequent negative outcomes.

Retaining Access and Restoring Exclusivity (RARE) Act (118th Congress: H.R.7383/S. 1214) – When the Orphan Drug Act (ODA) of 1983 originally passed Congress, it signified a monumental step forward for children with cancer and other rare disease patients. Over the past four decades, the incentives provided by the ODA have contributed to substantial growth in childhood cancer drug development. Unfortunately, the longstanding FDA interpretation of the ODA has been threatened due to a recent court decision[1]. If left unaddressed, this could have far-reaching adverse impacts on children with cancer and other rare diseases. The bipartisan RARE Act would codify Congress' interpretation of the ODA to ensure childhood cancer research and development isn't locked out from newly approved drugs that don't impact pediatric populations. In the 119th Congress, the RARE Act was also included in H.R 1262, the Give Kids a Chance Act.

Give Kids a Chance Act (H.R. 1262) – Children with relapsed cancer require time-sensitive clinical trials. For many children experiencing a relapse, a clinical trial can often be the best treatment available. Of the children whose relapsed cancer is cured, it is rarely due to just one drug. Many of these potentially life-saving trials require a combination of multiple drugs to improve outcomes, but due to regulatory hurdles, there are far fewer studies in children than adults. The Give Kids a Chance Act would better allow researchers to study combinations of new cancer drugs, potentially unlocking new cures for kids. In the 119th Congress, the Creating Hope Reauthorization Act, the RARE Act, and provisions of the Innovation in Pediatric Drugs Act were added to the bill, addressing many pressing needs in childhood cancer research.

Appropriations Priorities

For children with cancer, federally funded intramural and extramural research is the doorway to new, less toxic treatments. Due to the smaller patient populations, drug companies do not have as strong an incentive to invest in new childhood cancer research and development as they do for their adult counterparts. The status quo often favors existing childhood cancer treatments, despite their long-term health impacts. As a result, the onus is on the federal government to fill the gap. Research institutions around the country rely on grants from the National Institutes of Health (NIH) and the National Cancer Institute (NCI) to find new discoveries, treatments, and cures. The critical and innovative intramural research being done on campus at NIH and NCI has saved countless lives and must continue to be fully funded and unimpeded in its mission to improve the lives of childhood cancer patients, survivors, and families. **We join with the leading national cancer organizations in requesting at least $51.3 billion for NIH, including $7.934 billion**

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[1] *Catalyst Pharms., Inc.* v. *Becerra* (*Catalyst*), 14 F.4th 1299 (11th Cir. 2021)

for cancer research at NCI. Further, we strongly oppose any changes to NIH or NCI that threaten their long history of stable, uninterrupted, and robust support. Children cannot afford to wait.

The Childhood Cancer Survivorship, Treatment, Access, Research (STAR) Act is the most comprehensive and successful childhood cancer bill ever passed and reauthorized by Congress. It expands opportunities for childhood cancer research, improves efforts to identify and track incidences of childhood cancer, and enhances the quality of life for childhood cancer survivors. The programs authorized under the Childhood Cancer STAR Act serve as the critical infrastructure that makes childhood cancer research possible. To build on this incredible work, Congress must continue to make robust targeted investments to bolster childhood cancer survivorship research and expedite the discovery of new, less toxic treatments for the thousands of children who receive a cancer diagnosis each year. **We urge Congress to provide $30 million, the same funding level as last year, for this critical program.**

Initially created by President Trump in 2019, the Childhood Cancer Data Initiative is enhancing childhood cancer care and research data to improve preventive measures, treatment, quality of life, and survivorship, as well as ensure that researchers learn from every child with cancer. By building a community centered around childhood cancer care and research data, CCDI is making data more accessible for researchers and fast-tracking advances for children with cancer. With a primary goal of gathering data from every child, adolescent, and young adult diagnosed with a childhood cancer, CCDI is benefiting children diagnosed with cancer today and will lead to meaningful discoveries in the years to come. **We urge Congress to provide $50 million, the same funding level as last year, for this critical program.**

Since 2009, the Department of Defense's Peer Reviewed Cancer Research Program (PRCRP) has supported innovative research in cancers designated by Congress as relevant to those in military service and their families. Childhood cancer, the leading disease killer of children, adolescents, and young adults (AYAs) in the United States, affects families without prejudice regardless of geographical location, occupation, or income and impacts the military readiness of our service men and women. Childhood, adolescent, and young adult cancers differ in biology and types from older adults and, therefore, require targeted research. The lives of many children, adolescents, and young adults depend on vigorous and innovative research programs like PRCRP to enhance understanding of these deadly tumors and discover new treatments that are more effective and less toxic. **We urge Congress to maintain the same funding level as last year and continue including pediatric, AYA cancers, pediatric brain tumors, neuroblastoma, sarcomas, germ cell cancers, blood cancers, lymphoma, and thyroid cancer as eligible funding topics under the program.**

Other Immediate Priorities

Finally, Medicaid and CHIP provide quality, affordable healthcare coverage for over 80 million people, including over 37 million children, or roughly half of all children in the US. For children with cancer, Medicaid plays an especially critical role as a safety net. In many states, a child is eligible for Medicaid and CHIP coverage upon receiving a childhood cancer diagnosis, emphasizing the need for timely access to quality, uninterrupted care. Research has shown that

patients who experience disruptions in their Medicaid coverage are more likely to have advanced-stage disease and worse survival rates than patients without disruptions[2]. Survival rates have also been shown to improve in Medicaid expansion states relative to non-expansion states, particularly those living in lower-income areas[3]. **Our organizations strongly oppose changes to the Medicaid program that would restrict access, cut needed funding to states, create burdensome red tape, or reduce the quality or availability of services for children or their families.**

Thank you for your leadership on behalf of children with cancer. We look forward to working with you to improve the lives of childhood cancer patients, survivors, and families. Should you have any questions or need additional information, please contact Rosalie Abbott, Co-Chair of the Alliance for Childhood Cancer, at Rosalie.abbott@stbaldricks.org, or Dr. Michael Link, Co-Chair of the Alliance for Childhood Cancer, at mlink@stanford.edu.

Sincerely,

The Alliance for Childhood Cancer

American Cancer Society Cancer Action Network
American Childhood Cancer Organization
American Society of Pediatric Hematology/Oncology
The Andrew McDonough B+ Foundation
Association for Clinical Oncology
Association of Pediatric Hematology/Oncology Nurses
Association of Pediatric Oncology Social Workers
Children's Brain Tumor Foundation
Children's Cancer Cause
Dana-Farber Cancer Institute
The Leukemia & Lymphoma Society
Mattie Miracle Cancer Foundation
MIB Agents Osteosarcoma
National Brain Tumor Society
Pediatric Brain Tumor Foundation
Rally Foundation for Childhood Cancer Research
St. Baldrick's Foundation
St. Jude Children's Research Hospital

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[2] Xin Hu et al., Association Between Medicaid Coverage Continuity and Survival in Patients With Newly Diagnosed Pediatric and Adolescent Cancers. JCO Oncol Pract 0, OP.24.00268 DOI:10.1200/OP.24.00268

[3] Barnes JM, Neff C, Han X, Kruchko C, Barnholtz-Sloan JS, Ostrom QT, Johnson KJ. The association of Medicaid expansion and pediatric cancer overall survival. J Natl Cancer Inst. 2023 Jun 8;115(6):749-752. doi: 10.1093/jnci/djad024. PMID: 36782354; PMCID: PMC10248835.