December 10, 2024

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

The Honorable Mitch McConnell
Republican 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 Schumer, Republican Leader McConnell, 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 make key investments in research and take action on vital legislation affecting children with cancer before the conclusion of the 118th 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 had 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.

Appropriations Priorities

The Childhood Cancer STAR Act is the most comprehensive and succesful childhood cancer bill ever passed 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 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. **Increased funding, such as the $50 million included in both the House and Senate Labor, Health and Human Services,**

Education, and Related Agencies bills, would make a world of difference for patients, survivors, and their families.

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. **A $25 million increase, as included in the House's Labor, Health and Human Services, Education, and Related Agencies bill, will expand CCDI's impact and allow for further research in childhood cancer, getting us that much closer to new treatments and cures**.

Authorizing Priorities

For children with complex medical needs such as cancer, patients are routinely required to travel out of state to receive care or to 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 an out-of-state 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.

H.R. 4758/S. 2372, the Accelerating Kids' Access to Care Act (AKACA) 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. The 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. This September, AKACA was unanimously passed by the House of Representatives, and **we urge Congress to include the Accelerating Kids' Access to Care Act in any end of year legislative package.**

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, with the exception of certain oncology drugs. Further, under PREA, drug companies are required to study adult drug indications in children when children could benefit from pediatric studies. While sponsors are permitted to 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 (CMP's) for late post-market study requirements for adults, but the orphan drug exemption under PREA forbids FDA from doing the same for children.

H.R. 6664/S. 4905, the Innovation in Pediatric Drugs Act of 2023 amends the Pediatric Research Equity Act (PREA) to also remove the orphan drug exemption for all drugs and address the inequity in levying CMP's by giving FDA the authority to ensure post-market pediatric 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 September, and **we urge Congress include this in any end of year legislative package**.

The Creating Hope Reauthorization Act, originally passed in 2012 and reauthorized through 2024, expanded the FDA priority review voucher program to incentivize pharmaceutical manufacturers to invest in drugs with indications for rare pediatric diseases. Under this 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. **We urge Congress to reauthorize this important program, as unanimously passed by the House of Representatives in September**.

H.R. 3391/S. 1624, the Gabriella Miller Kids First Act 2.0 will build upon the successes of the original Gabriella Miller Kids First Act. Further, this legislation will expand the Kids First Program, which supports collaborative research to uncover the genetic etiology of childhood cancer and structural birth defects. The Kids First Program holds promise to facilitate more refined diagnostic capabilities and, ultimately, more targeted therapies or interventions. The Gabriella Miller Kids First Act 2.0 authorizes continued resources to improve treatment for childhood cancer by advancing the research that brings us closer to finding a cure. Legislation to reauthorize this important program was passed by the House of Representatives earlier this year, and **we urge Congress to reauthorize the Kids First Program before it expires at the end of the year**.

Thank you for your leadership on behalf of children with cancer. We look forward to working with you on these critical bills as we near the end of the 118th Congress. 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 of Pediatric Hematology Oncology Nurses

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

The Pediatric Brain Tumor Foundation

Rally Foundation for Childhood Cancer Research

St. Baldrick's Foundation