January 29, 2024

The Honorable Anna G. Eshoo
U.S. House of Representatives
272 Cannon House Office Building
Washington, DC 20515

The Honorable Michael McCaul
U.S. House of Representatives
2300 Rayburn House Office Building
Washington, DC 20515

Dear Representative Eshoo and Representative McCaul:

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 offer our endorsement of H.R. 6664, the Innovation in Pediatric Drugs Act of 2023. Thank you for your leadership in reducing key childhood cancer drug development barriers.

Approximately 1 in 263 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.

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. 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, maximizing the delivery of potential therapies to the children who need them most.

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. In 2012, PREA was amended to require FDA to issue and publicly post noncompliance letters to companies that have failed to submit their assessments on time. This effort has unfortunately not resulted in the completion of delinquent pediatric studies, and many required studies are still outstanding after being years overdue.

Current law allows FDA to assess civil monetary penalties 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 of 2023 would address this inequity by giving FDA the authority to penalize companies that do not complete their required pediatric studies. Failure to give FDA the authority it needs to ensure PREA studies get completed will jeopardize pediatric cancer studies required under the Research To Accelerate Cures and Equity (RACE) for Children Act, which went into effect in 2020.

Finally, the Innovation in Pediatric Drugs Act of 2023 provides needed funding increases for the Best Pharmaceuticals for Children Act (BPCA) NIH program. The BPCA NIH program funds studies of off-patent drugs that require further research in children. The program has been flat funded since 2002. This increase will ensure that the program is able to keep up with the rising costs of biomedical research inflation and continue its important work for children who too often rely on therapies that are decades old.

Thank you for your leadership on behalf of children with cancer. The Alliance for Childhood Cancer welcomes the opportunity to further discuss the unique challenges of childhood cancer drug development and research. We look forward to working with you as the Innovation in Pediatric Drugs Act of 2023 moves through the legislative process. 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,

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