CAC2
COALITION AGAINST
CHILDHOOD CANCER

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April 30, 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 and individuals are members of the Coalition Against Childhood Cancer (CAC2), whose members care deeply about all aspects of childhood cancer. We write in support of H.R. 6664, the Innovation in Pediatric Drugs Act of 2023. Your bipartisan legislation will help support children with complex medical needs, such as childhood cancers. Thank you for your leadership in reducing key childhood cancer drug development barriers.

Approximately 1 in 257 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—1 in 5 children diagnosed with cancer in the U.S. will not survive. Those who do survive often carry a heavy burden of their disease and treatment. By the age of 45, 95% of survivors have had a chronic health problem or have experienced a severe or life-threatening condition caused by the toxicity of the treatment that initially saved their life. These effects include brain damage, loss of hearing and sight, heart disease, secondary cancers, learning disabilities, and infertility.

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

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non-compliance 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 Coalition Against 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 Angela Lee, National Advocacy Lead at CAC2 (advocacy@cac2.org) or Vickie Buenger, President Emeritus at CAC2 (presidentemeritus@cac2.org)

Sincerely,

Coalition Against Childhood Cancer

CAC2 Organization Members

A Moment of Magic
Aiden's Army
Amanda Hope Rainbow Angels
Arms Wide Open Childhood Cancer Foundation
Bear Necessities Pediatric Cancer Foundation
Bearing Hope

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Braden's Hope for Childhood Cancer
Brooke's Blossoming Hope for Childhood Cancer Foundation
CancerFree KIDS
Carson Leslie Foundation
Children's Cancer Cause
Christina Renna Foundation Inc
Christina Renna Foundation Inc
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Elaine Roberts Foundation
End Kids Cancer
Friends of Cathryn Foundation
HelloCure
Joey's Wings Foundation
Julia's Grace Foundation
JUST TRYAN IT
Kids Get Cancer Too
Lilabean Foundation
MACC Fund
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Mighty Millie Foundation
Mystic Force Foundation
Neev Kolte & Brave Ronil Foundation
Neuroblastoma Children's Cancer Society
Northwest Indiana Cancer Kids Foundation
Oncoheroes Biosciences Inc
Our Amazing Fighters
Pediatric Brain Tumor Foundation
People Against Childhood Cancer (PAC2)
Pine Tree Apple Classic Fund
PREP4Gold
Rally Foundation for Childhood Cancer Research
Rett's Roost
Saving Sophie Foundation
SebastianStrong Foundation

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The Bardo Foundation
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The Morgan Adams Foundation
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The Scott Carter Foundation
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This Star Won't Go Out Inc
Ty Louis Campbell Foundation
WITH Grace Initiative
Zoé4life
Zoefia Alexandria Foundation

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