February 24, 2025

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

Dear 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. 1262, the Give Kids a Chance Act of 2025. Thank you for your leadership in reducing key childhood cancer research drug development barriers.

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.

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. In recent decades, we have seen dozens of new Food and Drug Administration (FDA)-approved cancer therapies; only a handful of treatments for childhood cancers have been approved over the same period. Despite the significant unmet medical need, pharmaceutical companies have been reluctant to develop drugs for childhood cancer, since the high costs associated with their research, development, marketing, and distribution are unlikely to be recouped following approval. The Creating Hope Act, which was originally passed in 2012, was subsequently reauthorized on a bipartisan basis. Unfortunately, due to Congressional inaction, the program expired at the end of 2024. The Creating Hope Act expanded the FDA priority review voucher program to incentivize pharmaceutical manufacturers to invest in drugs with indications for rare pediatric diseases. Through this program, FDA has awarded vouchers that offer new hope for children with cancer. The Give Kids a Chance Act of 2025 would reauthorize the rare pediatric disease priority review voucher program until 2029.

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 are permitted to request deferrals for their pediatric study commitments, FDA's existing authorities to enforce these deadlines have proven insufficient, as many required post-market pediatric studies are still delinquent years later. Current law allows FDA to assess civil monetary penalties for late post-market study requirements for adults, but exemptions under PREA forbid FDA from doing the same for children. Further, for many drugs that have been long off-patent, research is still needed in children. Unfortunately, the program that is tasked with this critical research, the Best Pharmaceuticals for Children Act (BPCA) NIH program, has been flat funded since 2002. The Give Kids a Chance Act of 2025 would give FDA the resources it needs to ensure PREA studies get completed on time and provide needed funding increases to the BPCA NIH program.

Since 1983, when the Orphan Drug Act (ODA) was enacted into law, Congress has interpreted that ODA exclusivity only applies to the approved indication within a rare disease or condition rather than the initial designation. 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 Give Kids a Chance Act of 2025 would codify Congress' interpretation of the ODA to ensure that childhood cancer research and development isn't locked out from newly approved drugs that don't impact pediatric populations.

Finally, 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.

Thank you for your leadership on behalf of children with cancer. We look forward to working with you to enact the Give Kids a Chance Act of 2025. 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

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

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