GIVE KIDS A CHANCE ACT OF 2025

- The FDA priority review voucher program, which was expanded in 2012 to incentivize pharmaceutical manufacturers to invest in drugs with indications for rare pediatric diseases, expired at the end of 2024 and must be reauthorized by Congress.
- Current law allows FDA to assess civil monetary penalties for late post-market study requirements for adults, but exemptions under the law forbid FDA from doing the same for children.
- The longstanding FDA interpretation of the Orphan Drug Act has been threatened due to a recent court decision, which could prevent some new treatments from being studied in children.
- Many 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.
- Please cosponsor **H.R. 1262/S. 932, the Give Kids a Chance Act of 2025**, which would address some of the most pressing research 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 guaranteeing pediatric studies happen in a timely manner.
- This legislation unanimously passed the House and was included in the bipartisan health title of the end-of-year package during the 118th Congress.

There are close to 7,000 rare diseases without appropriate treatments, and the vast majority of orphan diseases affect children. Despite the significant unmet medical need for new FDA-approved childhood cancer therapies, 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. In 2012 the FDA priority review voucher program was expanded and subsequently reauthorized on a bipartisan basis 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. The Give Kids a Chance Act of 2025 would give FDA the resources it needs to ensure PREA studies get completed on time.

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.

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.

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